

DEPARTMENTS OF LABOR, HEALTH AND HUMAN
SERVICES, EDUCATION, AND RELATED AGENCIES
APPROPRIATIONS FOR 2015

HEARINGS
BEFORE A
SUBCOMMITTEE OF THE
COMMITTEE ON APPROPRIATIONS
HOUSE OF REPRESENTATIVES
ONE HUNDRED THIRTEENTH CONGRESS
SECOND SESSION

SUBCOMMITTEE ON THE DEPARTMENTS OF LABOR, HEALTH AND
HUMAN SERVICES, EDUCATION, AND RELATED AGENCIES

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**DEPARTMENTS OF LABOR, HEALTH AND
HUMAN SERVICES, EDUCATION, AND RE-
LATED AGENCIES APPROPRIATIONS FOR
2015**

THURSDAY, FEBRUARY 27, 2014.

**OVERSIGHT HEARING—PUBLIC HEALTH EMERGENCY
MEDICAL COUNTERMEASURE ENTERPRISE**

WITNESSES

**GEORGE W. KORCH JR., PH.D., SENIOR SCIENCE ADVISER TO THE AS-
SISTANT SECRETARY FOR PREPAREDNESS AND RESPONSE**

**ROBIN ROBINSON, PH.D., DIRECTOR, BIOMEDICAL ADVANCED RE-
SEARCH AND DEVELOPMENT AUTHORITY**

**GREG BUREL, ACTING DEPUTY DIRECTOR, OFFICE OF PUBLIC
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**LUCIANA BORIO, M.D., ASSISTANT COMMISSIONER, COUNTERTER-
RORISM POLICY, FOOD AND DRUG ADMINISTRATION**

**MICHAEL KURILLA, M.D., DIRECTOR, OFFICE OF BIODEFENSE RE-
SEARCH RESOURCES AND TRANSLATIONAL RESEARCH, NATIONAL
INSTITUTES OF ALLERGY AND INFECTIOUS DISEASES, NATIONAL
INSTITUTES OF HEALTH**

Mr. KINGSTON. Okay. The committee will come to order and want to welcome everyone here.

I am going to start by welcoming Mr. Chris Stewart here, who is our newest committee member. And Chris, it is tradition of the new committee—number one, since you beat Mrs. Roby here, you can send her for your coffee. [Laughter.]

That is after you get the rest of us coffee. So that is the tradition, but we are glad to have both of you.

And I wanted to yield some time to my friend Rosa to welcome you all as well.

Ms. DELAURO. I do welcome both of you. It is a fabulous committee. I have had the opportunity to serve on it for a number of years. I think it is probably of the Appropriations subcommittees—I have to say for this and Agriculture because I serve on both of those committees—but it is really the place where there is such a broad spectrum of what affects people's lives every single day.

And so, it is a delight. I know you are going to enjoy the work, the camaraderie. And the chairman and I have had the opportunity to work together over the years not only in this committee, but in the Agriculture Subcommittee.

So I think it is a great place for you to be, and welcome. And you will learn a lot from this committee and listening to people like this this morning.

Thanks. Thank you, Mr. Chairman.

Mr. KINGSTON. Yes, I will say that this committee is just a deep well in all the various issues it gets involved in, and you can learn so much about so many things on it. And we have got a great staff. I am sure you have gotten familiar with them, and both the Democrat or the Republic side, they are there for you.

And most of the issues we deal with are not partisan at all. When you are trying to find a cure for a disease or something like that, you know, the science is the guide. So I think you will have a great time.

And with that, I wanted to welcome our witnesses today. This is our Public Health Emergency Medical Countermeasures Enterprise hearing, and it is the first hearing of the year. And our witnesses are Dr. George Korch, who is the senior adviser to the Assistant Secretary for Preparedness and Response; Dr. Robin Robinson, Director of the Biomedical Advanced Research and Development Authority, also which we all call BARDA; and Dr. Luciana Borio, who is with the FDA and the Assistant Commissioner for Counterterrorism Policy; and Dr. Michael Kurilla, with the National Institutes of Health in the area of allergy and infectious diseases; and Mr. Greg Burel, who is the Acting Deputy Director of the Office of Public Health Preparedness and Response, Centers for Disease Control.

So we are very happy to have all of you all here. And since we do have so many witnesses, we are going to ask you to keep your prepared comments to 3 minutes.

And then I want to say to the committee members, particularly Mrs. Roby and Mr. Stewart, we go by 5 minutes. And the first round of questions goes to who came here first, and then we just get in the regular order of the dais.

But we will stick strictly to the 5-minute rule. And Ms. DeLauro and I have been doing this for many years, both swapping the gavel back and forth. And we have always found that the shorter the questions, then you get more rounds in for everybody, and it is better.

So, with that, Dr. Robinson, the floor is yours.

Ms. DELAURO. Opening statement?

Mr. KINGSTON. Oh, yes, excuse me. And let me yield to Ms. DeLauro for an opening statement.

Ms. DELAURO. Thank you very much, Mr. Chairman. And as the chairman is wont to know, I do make opening statements.

And I only just have one correction in names, Mr. Chairman, and it is Luciana. Luciana Borio. [Laughter.]

I can't—I can't do all the pronunciations for everyone else, but this one I do know. So——

Mr. KINGSTON. Wait a minute. Wait a minute. So you are Italian? [Laughter.]

Ms. DELAURO. Ah, si.

Dr. BORIO. Sicilian background, but I go by Lu to make it simple for everybody. [Laughter.]

Mr. KINGSTON. Well, I am asking because Ms. DeLauro has always said next time we have an Italian witness, she is bringing lasagna, and—

[Laughter.]

Ms. DELAURO. You should have let me know that this was the case.

Mr. KINGSTON. We will have that happen. But we are glad to, of course, have anyone here, who is Brazilian or Italian, whatever.

And Dr. Korch, I understand we are going to start on the left and work our way over.

Ms. DELAURO. But, no.

Mr. KINGSTON. Oh, you haven't done your opening statement. Yes.

Ms. DELAURO. Thank you.

I want to thank our witnesses for their insights and their expertise that they will share with us this morning.

The subcommittee is evaluating our efforts to become better prepared with outbreaks of deadly diseases, particularly through development of new, better drugs and vaccines. Many of the efforts we will hear about today are aimed at limiting the harm from deliberate biological or chemical attacks, such as the spread of anthrax here in Washington 12 years ago. These programs were begun, greatly expanded in the last 10 or 12 years in response to growing recognition of serious gaps in our public health preparedness.

There have been some successes. Flu, for example. There was a time in the last decade when we were down to just one manufacturer of flu vaccine in the United States with only limited capacity to scale up production to respond to an epidemic. Today, we now have a much improved production capacity for the flu vaccine.

That being said, I think there are serious questions as to whether the vast resources that are dedicated to these programs are being spent in the most efficient manner to protect the public health. For example, we find ourselves 10 years into the BioShield program, having spent a whopping \$3,100,000,000, and we have to look at what do we show for that.

Certainly, an improved stockpile to deal with anthrax and smallpox, yet there is clearly a much wider spectrum of threats that confront us. We also need to be much better prepared to deal with emerging threats that occur naturally. Threats like the spread of novel diseases like SARS, the emergence of microbes that have become resistant to drugs used against them, and both pandemic flu and the ever-changing seasonal flu viruses.

I realize that BARDA has produced a broader range of products that are still in the development pipeline. But when these efforts were launched a decade ago, we expected to be further along by now. So I think our track record in developing medical countermeasures is decidedly mixed.

Just as important, we need to recognize that public health preparedness involves much more than simply developing and stockpiling drugs and vaccines. We also need enough well-trained epidemiologists, other health professionals to identify, investigate, and track disease outbreak. We need enough laboratory capacity to ana-

lyze large volumes of samples and determine what pathogens are involved.

We need effective plans, enough supplies and personnel to efficiently distribute and dispense vaccines and treatments. We need the surge capacity in our hospitals and other facilities to take care of large numbers of seriously ill patients.

All of this work needs to be done through partnerships between Federal agencies like CDC, State and local health departments, and the medical and first responder communities. Unfortunately, we have spent the past 5 to 10 years cutting Federal support for these critical State and local preparedness activities.

Adjusted to inflation, CDC funding to State and local health agencies has declined by nearly 50 percent in the past 10 years. Similarly, the Hospital Preparedness Program, which provides grants to States to improve the preparedness and resiliency of their healthcare system, has declined by about 60 percent.

These cuts cause State and local health departments to eliminate staff. They cut training exercises. They forgo critical medical equipment and technology. Addressing all these needs has become a real challenge for our subcommittee in light of the tight budget limits that are being imposed.

Much of the PHEMCE enterprise is really a new cost to this subcommittee that has to be fit within our constrained allocations. Until this year, all of BioShield and most of BARDA had been supported from a 10-year advance appropriation made back in 2004 in the homeland security bill. Much of the pandemic flu preparedness activity has been supported through balances of emergency supplemental appropriations made in 2006 and 2009.

However, now those funds have either expired, they are almost depleted, and this subcommittee has got to start covering the cost. That is \$800,000,000 in 2014 through annual appropriations.

What we had to do—and I want to say this to our panel as well as those of us up here—that unless we look at a different scale of allocation for this subcommittee, we had a serious shortfall in my view in the last allocation where we had to, because of the underfunding of some of the agencies that you represent here, we had to take on the \$800,000,000. And that had to come from someplace. It had to come from someplace. And it came from other areas.

These needs are all important. Investments provide tangible returns for the public. This subcommittee has got to take care of these issues. But with that tight constraint, it is going to be difficult to provide the adequate support to these countermeasure programs and take care of the many other public health priorities.

I will just give you one, the NIH. We saw only 58 percent of its sequestration cuts restored in the 2014 budget. And so, much of the basic scientific support for these efforts as well as other pieces at CDC and elsewhere necessary for public health preparedness will suffer the real, potentially grave consequences to the budget decisions that we make.

Weaker defenses against infectious diseases, slower progress in advancing medical science generally may be one of the consequences. So, today, I look forward to the discussion of both the current status of the PHEMCE programs and the challenges that we face ahead.

Thank you so much for joining us today, and I thank you, Mr. Chairman, for the time. We look forward to your testimony.

Mr. KINGSTON. Thank you.

And if there are no other opening statements, Dr. Korch.

DR. KORCH OPENING STATEMENT

Mr. KORCH. Chairman Kingston, Ranking Member DeLauro, and distinguished members of the committee, thank you for inviting me to testify on how the HHS PHEMCE is protecting the American public as a model for innovation and accountability in the Federal Government.

The Public Health Emergency Medical Countermeasure Enterprise, otherwise known as the PHEMCE, is the Federal coordinating body that oversees the lifecycle management of those medical countermeasures we rely on in the face of disasters arising from biological, chemical, radiological, or nuclear threats, or from novel and emerging pandemic diseases.

These include those drugs, vaccines, and other medical products the Congress and the administration have called for in past legislation to protect our population. Lifecycle management includes all aspects of the pipeline from initial research of promising products all the way through development, manufacture, purchase, stockpile, and distribution.

The PHEMCE assures that the talents and the authorities of a number of Federal agencies are well coordinated in this effort. The Assistant Secretary for Preparedness and Response leads the PHEMCE in partnership with the HHS agencies represented here today, as well as interagency partners from the Departments of Defense, Homeland Security, Agriculture, and Veterans Affairs.

Over the last 10 years, we have seen major returns on investment. We have produced and procured innovative medical countermeasures that will allow our Nation to better respond to medical and public health emergencies, ultimately saving lives and mitigating illness. This progress comes as a result of the support of the current and previous administrations, the Congress, and this subcommittee.

Above all, the PHEMCE mission, as stated in our strategic plan, demands that we preserve and protect people's lives against these major public health threats while exercising strong and effective stewardship of the taxpayers' dollars. The PHEMCE governance process and the decision framework aligns efforts, clearly articulates priorities, ensures that our resources are used effectively by all of our partners and for all segments of our population.

We have recognized the need to look at longer time horizons and to forecast our individual budget estimates in a cohesive way so that each partner understands the important handoffs and responsibilities across the lifecycle management of these medical products. We are addressing this through the multiyear budgeting process, a newly established tool the PHEMCE has devised to foster efficiencies and harmonization across the different stages of the medical countermeasure planning.

We also see the multiyear budget as an effective way to communicate the PHEMCE's commitment and priorities to our industry partners, who are key to the success of our program. With better

communication of priorities and resource needs, we are able to provide a higher degree of predictability to our partners and external stakeholders, including the Congress.

Coordinating resources and priorities of the partners that are represented here today is essential to establishing a sustainable and responsive medical countermeasure enterprise. This is foundational to our ultimate objective, a resilient nation prepared to respond and to recover from a wide range of those potential threats.

Thank you, and I look forward to your questions.

[The information follows:]



**Written Testimony
Committee on Appropriations
Subcommittee on Labor, Health and
Human Services
United States House of Representatives**

**The Public Health Emergency Medical
Countermeasures Enterprise**

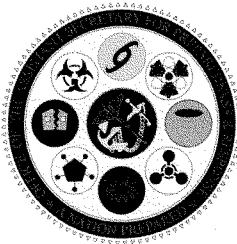
Statement of

George W. Korch Jr., Ph.D.

Senior Science Advisor

*Office of the Assistant Secretary for Preparedness and
Response*

U.S. Department of Health and Human Services



**For Release on Delivery
Expected at 10:00 a.m.
Thursday, February 27, 2014**

Chairman Kingston, Ranking Member DeLauro, and distinguished Members of the Committee, thank you for inviting me to testify on behalf of the Department of Health and Human Services (HHS) regarding the Public Health Emergency Medical Countermeasures Enterprise, also known as the PHEMCE.

Over the past ten years, we have made significant advances in the research, development, procurement, and distribution of medical countermeasures (MCMs) to address a wide range of naturally occurring and manmade threats, ranging from accidental releases to terrorism-induced. Congressionally supported initiatives such as the establishment of Project BioShield in 2004, the development of a national strategy to respond to pandemic influenza in 2005, and passage of the Pandemic and All-Hazards Preparedness Act in 2006 and its reauthorization in 2013, have provided valuable guidance and resources to address the potentially devastating consequences of a public health emergency. As a result of our efforts, twelve new, critical MCMs have been developed and delivered to the Strategic National Stockpile (SNS) including those for anthrax, smallpox, botulism toxin, and radiological and nuclear agents. These innovative countermeasures will allow our nation to better respond to emergencies, save lives, and mitigate illness. These achievements would not have been possible without federal interagency collaboration through the PHEMCE.

Established in 2006, the PHEMCE is the federal coordinating body that oversees the whole MCM lifecycle and assures that federal departments and agencies are not only coordinated, but working well together. The PHEMCE is led by the Office of the Assistant Secretary for Preparedness and Response (ASPR), in partnership with other HHS agencies, the National Institutes of Health (NIH), the Centers for Disease Control

and Prevention (CDC), and the Food and Drug Administration (FDA), and our interagency partners, the Department of Defense (DoD), the Department of Homeland Security (DHS), the Department of Veterans Affairs (VA), and the Department of Agriculture (USDA). Prior to the PHEMCE, federal efforts were fragmented and collaboration with our industry partners was limited. Through implementation of the 2007 and 2012 *PHEMCE Strategy and Implementation Plan* and the recommendations of the 2010 Secretary's Medical Countermeasure Review, the PHEMCE has become a model for innovative governance and accountable decision-making.

PHEMCE coordination and decision-making extends to encompass all stages of the MCM pathway, from identification of requirements for particular types and quantities of MCMs, through product development, and ultimately to distribution and use. All of these efforts are collaborative; agencies coordinate activities within their mission space and ensure smooth handoffs as products move from stage to stage. PHEMCE partners meet at least monthly to provide input into each stage of the process, develop plans and assessments, and centrally track implementation to ensure accountability. This level of coordination reduces duplication of effort and fragmentation. One notable example of this is the PHEMCE Integrated Portfolio for CBRN MCMs, which aligns the DoD and HHS advanced research and development programs across the civilian and military populations. This unprecedented collaboration allows both departments to pursue their distinct mission space while harmonizing overall objectives.

The lifecycle of a new MCM typically begins with risk assessment and requirements setting. DHS conducts the threat and risk assessments that result in Material Threat Determinations, which are required for procurement of MCMs under

Project BioShield; ASPR and other PHEMCE partners then use these assessments to establish MCM requirements. Based on these requirements, NIH shapes its investment strategy for new product research and discovery. Utilizing the early stage research funded by NIH and DoD, the Biomedical Advanced Development and Research Authority (BARDA, a component of ASPR) provides support to industry through advanced development programs. Once a product is ready, it moves forward for FDA approval to ensure that it is safe and effective. The culmination of this process is the stockpiling of FDA-licensed or approved products by the CDC in the SNS for use in an emergency. Once the products are delivered to the SNS, CDC develops operational plans to ship products to public health officials and clinical sites, where clinicians and responders will distribute them to individuals in need of medical care. These plans are integrated into the preparedness planning activities of all PHEMCE partners. In addition to the stockpiling and distribution through CDC, DoD and the VA procure and distribute products. Throughout, USDA works closely with HHS to address the zoonotic threats that may affect both animals and humans, such as highly pathogenic avian influenza.

The PHEMCE's ability to coordinate across the pipeline extends beyond our federal interagency to encompass public-private partnerships. As articulated in the Secretary's Review, the nation "must have the nimble, flexible capacity to produce MCMs rapidly in the face of any attack or threat;" this is only possible through close and continuing collaboration with our industry partners. The PHEMCE has promoted a number of innovative mechanisms for information sharing. For example, working with the FDA, we have clarified regulatory pathways and improved regulatory science to provide industry with the information they need to help move their products toward FDA

approval. All of our PHEMCE partners meet regularly with the private sector in a wide-range of forums, such as the annual BARDA Industry Day.

Above all, the PHEMCE must support the federal government's mission to preserve and protect people's lives against a wide range of dangerous threats and do so as good stewards of taxpayer dollars. The PHEMCE has thus developed a governance process and decision framework to ensure accountability and maximize the return on investment. PHEMCE leadership established criteria to ensure the maximum benefit to the health of the public, these include (1) addressing the most significant threats, (2) fostering approaches with the potential to provide protection against multiple important threats, and, (3) maintaining the capability to effectively use the assets developed in the envisioned operational setting.

As we look to the future, the PHEMCE continues to explore innovative ways to enhance the MCM pipeline and better serve the American public. The Pandemic and All-Hazards Preparedness Reauthorization Act of 2013 (P.L. 113-5) requires HHS to develop, and make available to Congress upon request, a five-year budget plan on the medical countermeasure enterprise. This multiyear plan is a tool for strategic project coordination, product transitions between agencies, communicating priorities and resources to partners stakeholders, and assisting with long-term forecasting..

The goal of the multiyear plan is to outline PHEMCE programmatic estimates on a five-year rolling basis and to identify the hand-offs in the development cycle in anticipatable budget terms. This forecast allows agencies to understand the dynamic effects of PHEMCE decisions on their own strategic planning and those of downstream partners. Forecasting can also inform the PHEMCE members of the effects of funding

on MCM availability in future years. For example, through a multiyear plan, the PHEMCE can forecast the programmatic goals of the SNS assuming various funding levels for Project BioShield. Additionally, this tool communicates the PHEMCE's commitment and priorities to our industry partners for their own planning purposes. By coordinating resources and priorities, we can ensure an active MCM industry that meets our essential needs for a nimble and flexible response capability.

The American people depend on HHS to protect against public health threats such as bioterrorism and emerging infectious diseases. A vibrant MCM enterprise, from end to end, is critical to our ultimate objective: a resilient nation prepared to respond to and recover from a wide range of potential threats. Since 2006, the PHEMCE has succeeded in establishing a strong pipeline of over 150 MCMs in all stages of development. Because of the PHEMCE's collaboration, communication, and accountability efforts, we now have a wider range of tools to address an ever-expanding list of threats but additional work remains. The PHEMCE is providing a roadmap for research, development, and procurement, to enable our federal and industry partners to prioritize resources and planning to fill those gaps. The true value of the PHEMCE to the nation may be in large-scale, public-private collaboration that enables us to evolve to meet the next treat, whatever that may be. Going forward, we will continue to refine our capacity and work towards meeting the expectations of Congress and the needs of the American people.

Mr. KINGSTON. Good job.
Dr. Kurilla.

DR. KURILLA OPENING STATEMENT

Dr. KURILLA. Thank you, Mr. Chairman and members of the committee. Thank you for the opportunity to discuss the role of NIH in the PHEMCE.

I am the Director of the Office of Biodefense Research Resources and Translational Research at the National Institute of Allergy and Infectious Diseases, known as NIAID, which is the component of the NIH with the lead for research on biodefense and emerging and reemerging infectious diseases. In fiscal year 2013, NIH funding for this research was approximately \$1,700,000,000.

The NIH supports foundational research towards the development of medical countermeasures against biological, chemical, radiological, and nuclear threats, including emerging and reemerging infectious diseases. NIH collaborations with its PHEMCE partners are critical and crucial to this endeavor. The NIH holds senior leadership positions within the PHEMCE, and NIH subject matter experts provide input to all PHEMCE working groups that coordinate efforts on particular biodefense threats.

NIH biodefense research aims to rapidly respond to manmade or naturally occurring threats, including microbes, toxins, chemical agents, radiation, and emerging or reemerging infectious diseases, such as seasonal and pandemic influenza. NIH transitions the advanced research and development of high-priority medical countermeasures to BARDA, with the eventual goal of FDA approval and possible inclusion within the SNS.

NIAID supports research and early-stage development of medical countermeasures as well as platform technologies to more rapidly and efficiently develop vaccines and diagnostics for a variety of threats. Our migration from a “one bug, one drug” approach towards a more flexible research paradigm is yielding advances that will enhance our ability to respond to the emerging public health threats of the future.

Recent successes in NIAID’s biodefense program include a next-generation smallpox vaccine and two smallpox antiviral drug candidates that have been transitioned to BARDA for further development. Other candidate products recently transitioned from NIAID to BARDA include therapies for anthrax and pandemic influenza. NIAID has also conducted studies that supported the first antibiotic approvals for pneumonic plague under the FDA’s “animal rule.”

Seasonal influenza and a potential emerging influenza pandemic remain serious public health challenges. NIAID supports research to develop medical countermeasures to diagnose, treat, and prevent seasonal and pandemic influenza. NIAID has collaborated with CDC, FDA, and BARDA to rapidly develop a vaccine for 2009 H1N1 pandemic influenza, and current efforts are focused on H7N9 avian influenza vaccine candidates. We are also working to develop a universal influenza vaccine, which could reduce the need for an annual vaccination and save millions of lives.

NIAID research also aims to understand the damaging effects of radiation and to develop medical countermeasures to diagnose and

treat radiation exposure. Twenty diagnostic—20 radiological candidates and 6 biodosimetry approaches supported by NIAID have been advanced to BARDA for further development, and NIAID has supported animal studies of Neupogen for the treatment of hematopoietic acute radiation syndrome.

The NIH remains committed to meeting public health emergency needs by advancing high-priority research for the development of medical countermeasures. Together with our PHEMCE partners, NIH will continue to pursue the development of diagnostics, therapeutics, and vaccines that will increase our national preparedness.

Thank you.

[The information follows:]

DEPARTMENT OF HEALTH AND HUMAN SERVICES
NATIONAL INSTITUTES OF HEALTH

**The Role of NIH Research
In The
Public Health Emergency Medical Countermeasures Enterprise**

Statement for the Record
House Subcommittee on Labor-HHS-Education Appropriations

Michael Kurilla, M.D., Ph.D.
Associate Director for Biodefense Product Development
National Institute of Allergy and Infectious Diseases

Mr. Chairman and Members of the Committee:

Thank you for the opportunity to discuss the role of the National Institutes of Health (NIH) in the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE). I am the Director of the Office of Biodefense, Research Resources, and Translational Research, and also serve as the Associate Director for Biodefense Product Development at the National Institute of Allergy and Infectious Diseases (NIAID). NIAID is the component of the NIH with the lead for research on biodefense and emerging and re-emerging infectious diseases. In fiscal year 2013, NIH funding for biodefense and emerging infectious diseases was approximately \$1.7 billion.

OVERVIEW

The September 11, 2001, terrorist attacks and anthrax attacks in the fall of 2001 underscored the importance of a national strategy for the development of medical countermeasures to combat bioterrorism and naturally emerging or re-emerging disease threats. To address this challenge, the NIH supports foundational research toward the development of new and improved medical countermeasures against biological, chemical, radiological, and nuclear threats, including emerging infectious diseases. NIH collaborations with its PHEMCE partners are critical to this endeavor. The NIH holds senior leadership positions within the PHEMCE, and participates throughout all its levels. For example, NIH subject matter experts provide input to all Integrated Program Teams (IPTs) that coordinate efforts on particular biodefense threats, and chair several IPTs.

Since 2001, NIAID has greatly accelerated its biodefense research program to rapidly respond to known and possible future threats, whether man-made or naturally occurring. NIH research addresses potential attacks using microbes, toxins, chemical agents, and radiation, as well as

naturally occurring emerging or re-emerging infectious disease threats such as seasonal and pandemic influenza. As part of this effort, NIAID conducts and supports basic research on microbiology and immunology; applied research to develop medical countermeasures; and clinical research to evaluate candidate diagnostics, therapeutics, and vaccines. NIAID coordinates with industry and PHEMCE partners to ensure that the results of NIAID-supported research can be translated rapidly into safe and effective medical countermeasures. In particular, NIAID transitions the advanced research and development of high-priority medical countermeasures to the Biomedical Advanced Research and Development Authority (BARDA) with the eventual goal of Food and Drug Administration (FDA) approval, licensure, clearance, or authorization and possible inclusion in the Strategic National Stockpile.

MEDICAL COUNTERMEASURES DEVELOPMENT:

RECENT ACTIVITIES AND ADVANCES

Biodefense and Emerging and Re-emerging Infectious Diseases. NIAID supports research and early-stage development of medical countermeasures against bioterror threats and emerging and re-emerging infectious diseases of public health importance. NIAID maximizes its efforts by pursuing development of medical countermeasures with wide impact, such as broad-spectrum antibiotics and antiviral drugs effective against multiple bacteria or viruses. NIAID also seeks to establish and validate efficient platform technologies to more rapidly develop vaccines and diagnostics for a variety of threat agents. NIAID's migration from a "one bug, one drug" approach toward a broader, more flexible research paradigm is yielding scientific advances that will facilitate public health emergency preparedness and our ability to respond to emerging public health threats.

Recent successes in NIAID's biodefense program include early-stage development of a next-generation smallpox vaccine. This vaccine would be suitable for populations, such as immunocompromised individuals, for whom the currently licensed smallpox vaccine is contraindicated. NIAID is currently exploring the use of this vaccine as a potential platform to facilitate the development of other vaccines. This next-generation smallpox vaccine and two smallpox antiviral drug candidates have been transitioned to BARDA for further development. Other candidate products recently transitioned from NIAID to BARDA include therapies for anthrax and pandemic influenza, and a next-generation anthrax vaccine. NIAID has conducted a study of the currently licensed anthrax vaccine to inform policy makers on strategies to extend vaccine supply should a shortfall occur. NIAID also successfully conducted studies that supported the first antibiotic approvals for pneumonic plague, a form of plague that causes severe and deadly lung infections, under the FDA's Animal Rule.

Influenza. Seasonal influenza and the possibility of an emerging influenza pandemic remain a serious public health challenge. The Centers for Disease Control and Prevention (CDC) estimates that seasonal influenza causes up to 49,000 deaths in the United States annually. Worldwide, the World Health Organization estimates that each year seasonal influenza causes up to 500,000 deaths.

NIAID supports and conducts research to better understand this global threat and develop medical countermeasures to diagnose, treat, and prevent seasonal and pandemic influenza. NIAID's efforts span the product development pipeline from basic research to understand influenza pathogenesis through discovery and testing of improved influenza antiviral therapeutics and vaccines. NIAID influenza research contributes to preparedness for emerging strains and potential pandemics. For example, NIAID collaborated with CDC, FDA, and

BARDA to rapidly develop a vaccine for 2009 H1N1 pandemic influenza. More recently, NIAID efforts have focused on the evaluation of H7N9 avian influenza vaccine candidates. In addition, NIAID is leading efforts to develop a so-called “universal” influenza vaccine to protect against multiple strains of seasonal and pandemic influenza. Such a vaccine could do away with the need for an annual vaccination and potentially save millions of lives and benefit global economic productivity.

Radiological/Nuclear and Chemical Threat Agents. NIAID research aims to understand the effects of radiation on the body, such as damage to the bone marrow, gastrointestinal tract, and lungs, and to develop medical countermeasures to diagnose and treat radiation exposure. To date, twenty radiological/nuclear medical countermeasure candidates and six biodosimetry approaches supported by NIAID have advanced to BARDA for further development. In addition, NIAID has funded animal efficacy studies of Neupogen for the treatment of hematopoietic acute radiation syndrome. A supplemental Biologics License Application for FDA review is in preparation.

The NIH medical chemical defense program, a trans-NIH partnership that includes NIAID, the National Institute of Neurological Disorders and Stroke (NINDS), the National Institute of Environmental Health Sciences, and a number of other NIH Institutes and Centers, is closely coordinated with PHEMCE partners and supports research to enhance protection against exposure to dangerous chemicals including nerve agents, metabolic poisons, and toxic industrial chemicals. Notably, NINDS recently supported clinical trials demonstrating that the drug midazolam may be an effective treatment for exposure to nerve agents for adults, as well as children. The results of these trials provide crucial support for midazolam as a medical countermeasure against nerve agents. Medical countermeasures addressing other significant

chemical hazards are under development, including drugs to treat pulmonary complications and neurotoxic effects of these chemicals.

CONCLUSION

The NIH remains committed to meeting public health emergency needs by advancing high-priority research for the development of medical countermeasures to combat pathogens utilized as bioterrorism agents and naturally emerging or re-emerging disease threats and chemical and radiological agents. Together with our PHEMCE partners, the NIH will continue to pursue the development of diagnostics, therapeutics, and vaccines that will increase our national preparedness.

Mr. KINGSTON. Thank you.
Dr. Robinson.

DR. ROBINSON OPENING STATEMENT

Mr. ROBINSON. Good morning, Chairman Kingston, Ranking Member DeLauro, and other distinguished members of the subcommittee.

Thank you for the opportunity to speak with you today about Government biodefense efforts. I am Robin Robinson, the Director of BARDA, and Deputy Assistant Secretary for ASPR in HHS.

BARDA, like ASPR, was established by the Pandemic and All-Hazards Preparedness Act of 2006 and mandated to support advanced development and acquisition of novel and innovative medical countermeasures such as vaccines, therapeutics, diagnostics, and medical devices for the Nation to address the medical consequences of manmade threats and mother nature like the H1N1 pandemic.

Medical countermeasure development is risky, lengthy, and costly, with many inexperienced developers failing and larger pharmaceutical companies avoiding this sector completely. BARDA bridges the "valley of death" that prevents the transition of many product candidates from early development to commercialization and FDA approval by providing funding and technical core service assistance.

BARDA has made good on the mandate in a number of ways. First, BARDA has established a robust and formidable product development pipeline of more than 150 product candidates, including many for special populations like children. FDA has approved 7 first-in-class products supported by BARDA in the last 18 months.

From this pipeline, we have procured 12 novel products under Project BioShield to date, including smallpox vaccines and antiviral drugs, anthrax vaccines, and antitoxins which have been licensed, botulinum antitoxins which have been licensed, radionuclide chelators, anti-neutropenia cytokines for radiation illness, and chemical agent anticonvulsive drugs.

BARDA has also built and expanded domestic manufacturing capabilities for these medical countermeasures by establishing national stockpiles for H5N1 and H7N9 vaccines and new adjuvants for pandemic preparedness, created public-private partnerships with industry, and have expanded domestic pandemic influenza vaccine manufacturing surge capacity several fold and developed new classes of antimicrobial drugs for biothreats and prominent classes of antimicrobial drug-resistant pathogens like CRE and MRSA.

BARDA has also established new and innovative ways to help inexperienced companies through the Core Service Assistance programs. These are animal studies networks, Centers for Innovation in Advanced Development and Manufacturing, Fill Finish Manufacturing Network, and Clinical Studies Network that will be established this year.

Response capabilities from these Core Service Assistance programs have already been utilized for recent public events such as H7N9 outbreaks. BARDA has increased the sustainability of these biodefense preparedness by actually moving from a one bug, one

drug paradigm to actually repurposing existing drugs and developing multipurpose products that can be used for biotreats and also everyday public health emergencies.

BARDA has also embraced the multiyear budgeting, but we still have a number of obstacles to come. Complacency about threats is here today, and we must remain vigilant about that.

I look forward to answering your questions as we go forward and look forward to your continued support as we go forward in this endeavor.

[The information follows:]



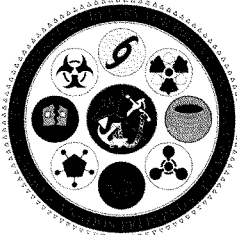
**Written Testimony
Committee on Appropriations
Subcommittee on Labor, Health and
Human Services
United States House of Representatives**

BARDA'S Role in the PHEMCE

Statement of

Robin A. Robinson, Ph.D.

*Deputy Assistant Secretary and BARDA Director
Office of the Assistant Secretary for Preparedness and
Response
U.S. Department of Health and Human Services*



**For Release on Delivery
Expected at 10:00 a.m.
Thursday, February 27, 2014**

Good morning. Chairman Kingston, Ranking Member De Lauro and other distinguished Members of the Subcommittee, thank you for the opportunity to speak with you today about government biodefense efforts. I am Dr. Robin Robinson, Director of the Biomedical Advanced Research and Development Authority (BARDA) and Deputy Assistant Secretary for Preparedness and Response (ASPR) at the Department of Health and Human Services.

Within the Public Health Emergency Medical Countermeasure Enterprise (PHEMCE), BARDA, like its parent organization, ASPR, was established by the Pandemic and All-Hazards Preparedness Act of 2006. Aligned with the PHEMCE strategy and implementation plans, BARDA is the government agency mandated to support advanced development and acquisition of novel and innovative medical countermeasures (MCM) such as vaccines, antimicrobial drugs, diagnostics, and medical devices for the entire nation to address the medical consequences of man-made threats and Mother Nature like the H1N1 pandemic.

Medical countermeasure development is risky, lengthy, and costly with many inexperienced developers failing and larger pharmaceutical companies avoiding the sector completely. BARDA, as a key partner in the PHEMCE, serves as a bridge over a critical gap referred to as the "Valley of Death" in MCM development through direct support, public-private partnerships, and technical core service assistance. Once the PHEMCE establishes product requirements NIH launches discovery and early stage development of product candidates from industry partners then transitioning to BARDA for advanced research and development support and assistance to reach sufficient maturity for product acquisition under Project BioShield, towards FDA approval and

eventual stockpiling at CDC's Strategic National Stockpile (SNS) or the commercial sector. Upon FDA approval, the financial responsibility of MCMs transfers from BARDA under Project BioShield to the SNS to stockpile and deliver. Finally, in public health emergencies BARDA takes a response role by interfacing with other PHEMCE partners and manufacturers to develop, produce, and test products for FDA review and approval and CDC distribution to state and local providers, as illustrated in the 2009 H1N1 pandemic and the H7N9 outbreaks.

BARDA has delivered on its founding mandate to develop products towards regulatory approval and acquire them for national security and public health preparedness.

Towards our primary strategic goal, we have become the bridge across the "Valley of Death" a gap in the pharmaceutical development pipeline that obstructs early development candidates from reaching commercial manufacturing capability and ultimate FDA approval. A lack of a commercial market and significant up front commercial investments prevent MCM developers from succeeding. BARDA support and funding facilitate successful product development and have established a robust and formidable product development pipeline of 150+ product candidates. BARDA has procured 12 novel products under Project BioShield since its inception and include smallpox vaccines and antiviral drugs, anthrax vaccines and antitoxins, botulinum antitoxins, radionuclide chelators, anti-neutropenia cytokines for radiation illness, and chemical agent anti-convulsive drugs. BARDA has built a national stockpile of H5N1 and H7N9 vaccines and new adjuvants for pandemic preparedness. Recently, FDA has approved 7 first-in-class products supported by BARDA.

BARDA has created public-private partnerships with industry partners to expand domestic pandemic influenza vaccine manufacturing surge capacity several fold by retrofitting or building new manufacturing facilities in the U.S. BARDA used Other Transactions Authority in 2013 to establish a unique partnership with industry to develop new classes of antimicrobial drugs for biothreats and prominent antimicrobial resistant pathogens such CRE and MRSA – part of BARDA's role in the national war on antimicrobial drug resistance.

Further BARDA has created Core Service Assistance programs to help inexperienced drug developers and provide MCM response capabilities. In 2010, BARDA enlisted 17 laboratories in the U.S. and the U.K. to develop qualified animal models and perform animal challenge studies on CBRN MCMs. In 2012, BARDA established 3 domestic Centers for Innovation in Advanced Development and Manufacturing (CIADM) to assist CBRN product developers routinely with the development and manufacture of products for clinical studies and stockpiling. In an influenza pandemic, these Centers will double as flexible manufacturing facilities rapidly producing millions of vaccine doses.

Recently, BARDA established a Fill Finish Manufacturing Network comprised of 4 domestic manufacturers who can aseptically fill pandemic influenza vaccines, assist the Centers with filling needs, and address drug shortages as appropriate. This year, BARDA plans to unveil a Clinical Studies Network comprised of multiple Clinical Research Organizations to address clinical needs of BARDA's product developers and support BARDA's response needs. Together these Core Service Assistance programs effectively mitigate the risk of drug development and make MCMs available in emergencies.

Lastly, BARDA has increased the sustainability of biodefense preparedness and readiness by changing our approach from a “one bug – one drug” paradigm to supporting development of existing drugs that may be repurposed such as anti-neutropenia drugs for radiation treatment and new drugs that have multiple indications including biodefense and commercial public health usages such as new antibiotics for biothreats and community- and hospital-acquired pathogens, especially CRE and MRSA.

Another new tool that the PHEMCE is using to streamline transition of product candidates to one another and address life-cycle management costs is multiyear planning as mandated in the Pandemic and All-Hazards Reauthorization Act. This tool tracks both financial resources and implementation of the PHEMCE Strategic Implementation Plan across PHEMCE partners. BARDA forecasts its product priorities and transitions to PHEMCE partners.

BARDA expects over the next 5 years to receive new product candidates for its advanced development pipeline that will treat viral hemorrhagic fever, multidrug resistant pathogens, and radiation illnesses and provide more effective influenza vaccines. BARDA expects 12 more new MCMs to mature sufficiently in advanced development for acquisition under Project BioShield; these MCMs include next generation anthrax vaccines, better smallpox vaccines, biodosimetry diagnostic devices, thermal burn radiation drug and skin replacement therapies, radiation cell therapies, new antibiotics, and new chemical antidotes, provided appropriations are available. Lastly, several MCMs that have been approved recently or will be approved soon by the FDA are expected to move from Project BioShield to the Strategic National Stockpile for

future acquisition; these include anthrax and botulinum antitoxins and smallpox vaccines and antiviral drugs. As multiyear budgeting improves within the PHEMCE, these transitions will become seamless and able to address life cost management more effectively.

The PHEMCE and BARDA still face formidable obstacles in the coming years including complacency towards biodefense threats; emergence of new threats, especially from Mother Nature; managing life cycle costs of existing and more new products; new regulatory pathways to traverse; and the uncertainty of funding to purchase new medical countermeasures under Project BioShield to maintain and enhance preparedness and readiness levels. BARDA will tackle these challenges with PHEMCE and industry partners by executing existing plans for building domestic manufacturing capacity and streamlining development costs through the CIADMs and working with FDA to establish regulatory management plans for product developers. In conclusion, I would like to reiterate that as a member of the PHEMCE, BARDA is a proven and reliable partner and is creating vital national assets that enhance national security and address public health preparedness and response needs. Again, I would like to thank the subcommittee for the opportunity to testify, and I look forward to your questions.

Mr. KINGSTON. Thank you.
Mr. Burel.

MR. BUREL OPENING STATEMENT

Mr. BUREL. Good morning, Chairman Kingston, Ranking Member DeLauro, and members of the subcommittee. Thank you for the invitation to be with you today.

Public health emergencies, such as the 2009 H1N1 influenza pandemic or 2012's Hurricane Sandy, can quickly overwhelm State and local public health resources. CDC provides scientific expertise, guidance, and support for State and local public health systems as they work to prevent, protect, mitigate, respond to, and recover from all public health emergencies that threaten the health of American people at home and around the world.

This wealth of expertise and the drive to innovate helps inform Federal decisions on responding to emerging threats, including those decisions on deployment of assets from the Strategic National Stockpile, the Nation's repository of medical countermeasures currently valued at approximately \$5,900,000,000.

CDC supports the PHEMCE by providing subject matter expertise and executive guidance, current information on the assets held by the SNS, expertise in the management and distribution of these assets to the States, and an understanding of State and local plans to dispense these medical countermeasures. CDC works with the PHEMCE to prioritize and address gaps in the highest-priority threat areas and transforms these recommendations into assets ready to protect lives through procurement of materials and management of those materials.

The SNS is a unique Federal asset, embedded in a public health threat detection and response agency. The rapid response to emergencies depends on robust State and local infrastructure and planning.

The Public Health Emergency Preparedness Cooperative Agreement, or PHEP, is a critical companion program to the SNS. This provides funding and expertise to our State and local partners to support their planning efforts to effectively receive and make use of medical countermeasures delivered from the stockpile.

These interconnected programs assured that during the H1N1 pandemic CDC was able to efficiently deploy stockpiled antiviral drugs and personal protective equipment to our partners throughout the Nation. It assured that Oregon was able to conduct a mass vaccination campaign to respond to a meningitis attack—or outbreak. And it assures that we can rush antitoxins and treatments to any person exposed to anthrax, botulism, or other public health threats.

As part of CDC's management responsibility, the SNS acquires commercially available pharmaceuticals, devices, and ancillary supplies. Besides outright purchases, CDC uses vendor-managed inventory and a number of other innovative purchase agreements based on the lowest cost to the U.S. Government over the entire lifecycle of a product.

Additionally, CDC is responsible for replacing SNS-held medical countermeasures licensed and/or initially procured by BARDA. CDC develops policies and guidance for the use of these medical

countermeasures and forward deploys them to respond quickly anywhere in the country.

We have always sought to maximize the effectiveness of resources and investments. We are even more so focused in the current fiscal environment. Efforts to maximize the impact of our national—this unique national resource include recent independent reviews by two Federal advisory committees and a forthcoming congressionally mandated review by the IOM.

I thank you again for the opportunity to testify, and I will be happy to answer any questions you may have.

[The information follows:]



Testimony
Subcommittee on Labor, Health and
Human Services, Education, and Related
Agencies
Committee on Appropriations
U.S. House of Representatives

Centers for Disease Control and Prevention
Support for the Public Health Emergency Medical
Countermeasures Enterprise

Statement of

Greg Burel

*Acting Deputy Director, Office of Public Health Preparedness and
Response*

*Centers for Disease Control and Prevention
Department of Health and Human Services*

For Release upon Delivery
Expected at 10:00 p.m.
Thursday, February 27, 2014

Introduction

Good morning, Chairman Kingston, Ranking Member DeLauro, and Members of the Subcommittee. I am Greg Burel, Acting Deputy Director of the Office of Public Health Preparedness and Response at the Centers for Disease Control and Prevention (CDC). Thank you for the invitation to address the Subcommittee today. My remarks will focus on CDC's role in strengthening our nation's public health preparedness and response through our many CDC programs, our participation in the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE), and our coordinated activities with partners in federal, state and local government as well as the private sector.

Background

Public health emergencies, such as the 2009 H1N1 influenza pandemic or 2012's Hurricane Sandy can quickly overwhelm state and local public health resources. In these and numerous other situations, our state and local partners rely on federal support and resources to execute their emergency response plans and ensure the health and safety of people in the United States. Because of its unique ability to support responses to infectious, occupational, and environmental incidents, CDC plays a pivotal role in ensuring that state and local public health systems are prepared for public health emergencies regardless of their nature – pandemics, natural disasters, or terrorism. The cornerstone of these efforts is supporting the resilience of public health systems used for routine threats so that they can scale up as needed. CDC's guidance and support for these public health systems is grounded in the agency's depth of experience and expertise combatting disease around the world and protecting the health of the American people.

CDC leads the federal government's activities related to public health surveillance, epidemiologic and laboratory investigations, public health communications, and delivery of medical countermeasures for public health emergencies. CDC resources help sustain the state and local public health infrastructure, and our expertise helps state and local partners develop their capabilities to prepare for and effectively respond to public health emergencies. CDC plays an integral role in public health surveillance and investigation by synthesizing and interpreting data to better evaluate and characterize public health emergencies. This information then helps inform federal decisions on whether to deploy assets from the Strategic National Stockpile (SNS), the nation's repository of medical countermeasures valued at approximately \$5.9 billion.

CDC provides subject matter expertise and executive guidance to the structured PHEMCE deliberation and decision-making process. Additionally, CDC supports PHEMCE with current information on the assets held within the SNS, expertise in the management and distribution of these assets to the States, and an understanding of state and local plans to dispense medical counter-measures (MCM). CDC works through the PHEMCE governance process to prioritize and address gaps in the highest priority threat areas. Finally, CDC has the responsibility to transform PHEMCE's recommendations into assets ready to protect lives through procurement of materials and management of the SNS.

CDC manages the SNS, including acquisition of commercially available pharmaceuticals, devices, and ancillary supplies to meet PHEMCE requirements for SNS. CDC also procures replacements for SNS-held MCMs licensed and/or initially procured by the Biomedical Advanced Research and Development Authority (BARDA) using the Special Reserve Fund.

CDC leads research into alternative methods of forward deployment of countermeasures and dispensing options. CDC also develops utilization policies and guidance for stockpiled medical countermeasures and provides guidance to state and local partners for planning and implementing mass distribution and dispensing activities.

CDC works to continually improve our capability to deliver SNS assets to affected areas during public health emergencies. In 2013, CDC implemented an interagency agreement with the Defense Logistics Agency (DLA) for procurement support. This new partnership, formalized in July 2013, continues the trend of improving SNS cost efficiency through strategic procurement partnerships. Access to existing DLA contract pricing and its procurement system is expected to yield reduced procurement costs and has already decreased delivery times for MCM orders by 75%..

Getting these life-saving products to the people who need them during an emergency depends on robust state and local infrastructure and planning. CDC goes beyond stockpiling and delivering SNS assets, by supporting our state and local partners in building, refining, and sustaining their abilities to effectively receive and use medical countermeasures delivered from the SNS. CDC supports these partnerships through the Public Health Emergency Preparedness (PHEP) cooperative agreement, which provides funding, and other resources to develop and improve distribution and dispensing capabilities. CDC is also exploring innovative ways to dispense these products to communities by cultivating strong collaborative partnerships among state and local planners, emergency responders, and businesses.

Budget

CDC's preparedness and response activities for the SNS are funded through direct appropriations of no-year funds. In FY 2014, CDC received an appropriation of \$535 million, up from \$477.6 million in FY 2013, for its SNS activities. This funding goes toward purchasing and maintaining MCMs designed to help the public respond to and recover from infectious disease outbreaks; chemical, biological, radiological, or nuclear terrorist events; and major natural disasters. Additionally, this funding allows CDC to provide training and consultation to support the ability of state, local, tribal, and territorial health departments to receive, organize, store, distribute, and dispense federal medical supplies.

Measuring Success

CDC has demonstrated the success of agency preparedness programs repeatedly in real world responses to public health emergencies. From the deployment of staff and MCM material to New York City in the hours following the 9/11 attacks to the deployment of antiviral drugs and personal protective equipment to every state and territory during the response to H1N1 in 2009, CDC has repeatedly proven its ability to deploy SNS assets in support of state and local response efforts. From each response, CDC has gained valuable experience, and has systematically identified opportunities to improve preparedness at the federal, state and local levels. Through the application of this experience, CDC has improved SNS distribution and dispensing capabilities, and has supported the development and improvement of state and local all-hazard response plans.

State and local responses have demonstrated the effectiveness of these all-hazard plans, and the impact of PHEP and SNS investments in building state and local response capacity for distributing and dispensing SNS assets that have been deployed. In addition, state and local jurisdictions have used their MCM response capabilities in other public health interventions, including support for populations displaced by flooding and implementation of mass vaccination campaigns.

Conclusions

The Strategic National Stockpile is a unique federal asset. Effectively using SNS assets requires collaboration among state, local, tribal, territorial, and federal partners on many aspects ranging from MCM product research to development of diagnostics to detection of an event to distribution and dispensing of medical countermeasures.

CDC has always sought to maximize the effectiveness of resources and investments, and is even more focused on doing so in the current fiscal environment. To that end, CDC continues to work with federal partners, including the Department of Homeland Security, to better integrate federal capabilities to identify, develop, acquire, distribute, and dispense MCM—with the ultimate goal of getting the most effective medical countermeasures to the people who need them when they need them.

I thank you again for the opportunity to testify before you today. I will be happy to answer any questions you may have.

Mr. KINGSTON. Thank you.
Dr. Borio.

DR. BORIO OPENING STATEMENT

Dr. BORIO. Good morning. Good morning, Chairman Kingston, Ranking Member DeLauro, subcommittee members. I am happy to appear before you and my public health colleagues.

I would not be here today if it were not for your strong leadership on this issue, Chairman Kingston and Ms. DeLauro. Three years ago, as the chair and ranking member of the Agriculture Appropriations Subcommittee, you approved bill language for the fiscal year 2011 appropriations act that launched the FDA program that I lead. And in subsequent years, you sustained or increased funding for FDA's Medical Countermeasures Initiative.

And now Chairman Aderholt and Representative Farr are continuing this support. So I thank you for your vision and your leadership. On behalf of myself and my colleagues at the witness table and on behalf of the Americans who may someday need new medical products to address CBRN threats and the Americans who will continue to benefit from advances related to pandemic influenza and other emerging infectious diseases, thank you.

FDA plays a critical role in protecting the U.S. from these threats. We ensure that countermeasures—drugs, vaccines, and diagnostic tests—are safe, effective, and secure. Collaboration with our interagency partners is the cornerstone of this critical public health responsibility.

FDA also partners with the Department of Defense to support the unique needs of the warfighter. FDA employs its authorities, such as the emergency use authorization, to facilitate access to available countermeasures to respond to public health emergencies even when products are not yet approved for use. We also provide scientific and regulatory counsel on stockpiling and deployment decisions.

The resources you approved allowed FDA to hire essential expert staff and become even more engaged in the countermeasure activities. With this increased engagement, we helped resolve many regulatory challenges and impediments associated with developing these highly complex medical products. We are working to ensure that their development does not stall.

Your investment facilitated the approval of several countermeasures, including a therapy for inhalational anthrax, a botulism antitoxin, a next-generation portable ventilator, and several influenza diagnostic tests and vaccines. We have readied countermeasures for potential use and our EUA authority against an array of threats, including smallpox, anthrax, pandemic influenza, and nuclear threats.

We worked closely with this Congress on the passage of the Pandemic and All-Hazards Preparedness Reauthorization Act of 2013, which created new authorities for the FDA, and we have already put these new authorities to good use. We were able to issue EUAs for diagnostic tests for the Middle East respiratory syndrome coronavirus and for the avian influenza H7N9 virus, including one requested by the CDC and another requested by a commercial developer.

With input from our enterprise partners, FDA has established a broad and robust regulatory science portfolio, which is essential for our regulatory decision-making.

I want to conclude by emphasizing that developing counter-measures is highly complex. Close cooperation is essential, and the deep engagement represented here today exemplifies public health synergy at its best. What we collectively achieve on behalf of the American public is far greater than the sum of our parts, and thank you for making this possible.

[The information follows:]



DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

Food and Drug Administration
Silver Spring, MD 20993

“HHS Agencies’ Efforts to Prepare the Nation to Combat Biological Events”

Statement of

Luciana Borio, MD

Assistant Commissioner for Counterterrorism Policy

Director, Office of Counterterrorism and Emerging Threats

Deputy Chief Scientist (Acting)

US Food and Drug Administration

Department of Health and Human Services

Before the

United States House of Representatives

Committee on Appropriations

Subcommittee on Labor, Health and Human Services, Education, and Related Agencies

February 27, 2014

Introduction

Good morning Chairman Kingston, Ranking Member DeLauro, and members of the Subcommittee. I am Dr. Luciana Borio, Assistant Commissioner for Counterterrorism Policy, Director of the Office of Counterterrorism and Emerging Threats, and Acting Deputy Chief Scientist at the Food and Drug Administration (FDA). Thank you for the opportunity to appear today to discuss FDA's efforts to prepare our nation to mitigate biological threats – such as a biological weapons attack or naturally emerging infectious diseases like pandemic influenza and antimicrobial resistant pathogens.

FDA's Medical Countermeasure Mission

FDA plays a critical role in protecting the United States from deliberate chemical, biological, radiological and nuclear (CBRN) threats and naturally occurring infectious diseases. Specifically, FDA is responsible for ensuring that medical countermeasures—including drugs, vaccines, and diagnostic tests—to counter these threats are safe, effective, and secure. The mission of my office is to facilitate the development and availability of these life-saving products.

Collaboration is the cornerstone of this critical public health responsibility. FDA works closely with its interagency partners through the Department of Health and Human Services (HHS) Public Health Emergency Medical Countermeasures Enterprise (Enterprise) to build and sustain the medical countermeasure programs necessary to respond effectively to public health emergencies. FDA also works closely with the U.S. Department of Defense (DoD) to facilitate the development and availability of medical countermeasures to support the unique needs of the warfighter. For example, FDA supports the Enterprise and DoD by providing subject matter expertise for developing medical countermeasures. We also provide scientific and regulatory counsel to inform medical countermeasure stockpiling and deployment decisions. In addition, FDA employs its authorities, such as Emergency Use Authorization (EUA), to facilitate access to medical countermeasures to respond to public health and military emergencies, even when products are not yet approved for any use or the particular use needed.

In 2010, FDA launched its Medical Countermeasures initiative (MCMi), focusing increased resources on identifying and resolving regulatory challenges to medical countermeasure development and availability. Within FDA, MCMi promotes the development of medical countermeasures by establishing clear regulatory pathways for medical countermeasures, instituting effective regulatory policies and mechanisms to facilitate timely access to available medical

countermeasures, and advancing medical countermeasure regulatory science to create the tools that support regulatory decision-making.

Launching MCMi at FDA

Just prior to the close of FY 2010, FDA received \$170 million in one-time, no-fiscal year funding to immediately commence MCMi activities. FDA used these resources to establish the MCMi program, hire 77 FTEs and conduct regulatory science activities to address and resolve important questions related to medical countermeasures. Between FY 2011 and FY 2013, FDA expended \$126.3 million of its no-year funding for this purpose, and we expect to spend the remaining \$12.2 million on related research during FY 2014 and FY 2015.

Between FY 2012 and FY 2014, FDA received core funding from Congress to sustain and modestly expand its MCMi program. For FY 2012, FDA received an appropriation of \$20.0 million to support MCMi activities that we funded in previous fiscal years with one-time money. The FY 2012 appropriation allowed FDA to sustain 70 of its 77 MCMi FTEs and support a \$327,000 investment in MCM regulatory science. In FY 2013, Congress increased FDA's budget for MCMi by \$3.5 million, providing a total MCMi base of \$23.5 million. The sequestration and rescission diminished some of the MCMi FY 2013 budget increase. However, the FY 2013 funding level allowed FDA to support the 77 FTE initially hired with no-year funds as well as increase our annual investment in MCM regulatory science to \$1.4 million. In FY 2014, Congress provided FDA with a \$1.0 million increase for MCMi, for a total base of \$24.5 million. FDA will primarily use this increase to support FTE costs.

Scope of MCMi Operations

FDA's scope of operations within the medical countermeasures responsibilities covers a broad range of activities essential to facilitating development and access to safe and effective medical countermeasures, including:

- Reviewing medical countermeasure marketing applications and approving those that meet applicable standards for safety and efficacy;
- Providing regulatory advice, guidance and – when needed – technical assistance to medical countermeasures product sponsors and our Enterprise partners;
- Supporting efforts to establish and sustain an adequate supply of medical countermeasures, including averting supply disruptions when feasible and, in certain situations, allowing products to be used beyond their expiration dates when supported by our scientific analyses;

- Supporting the development of advanced manufacturing technologies by collaborating with the HHS Biomedical Advanced Research and Development Authority (BARDA) on their Centers for Innovation in Advanced Development and Manufacturing;
- When necessary, enabling access to medical countermeasures that are not yet approved for use through an appropriate mechanism, such as EUA;
- Ensuring that FDA regulations and policies adequately support medical countermeasures development and enable preparedness and response activities;
- Fostering the professional development of our scientists to ensure that FDA personnel maintain the skills and abilities to support the medical countermeasure mission;
- Proactively identifying and resolving regulatory challenges associated with medical countermeasures; and
- Improving medical countermeasure development timelines and success rates through an MCMi Regulatory Science Program that harnesses cutting-edge science and applies innovative approaches to answer questions that are vital to FDA regulatory decisions.

Measures of Success

The additional resources that Congress provided to FDA for the MCMi have enabled FDA to hire expert staff and become more deeply and thoroughly engaged in medical countermeasure activities throughout the Enterprise. With this increased engagement, FDA has helped to resolve many regulatory challenges and impediments associated with the U.S. government's advanced development medical countermeasure pipeline so that medical countermeasure development does not stall and continues to move forward. For example, this has resulted in the recent approval, licensure, or clearance of several medical countermeasures, including an inhalational anthrax therapeutic, a botulism antitoxin, a next-generation portable ventilator, and several influenza diagnostic tests. Of note, FDA was able to approve the inhalational anthrax therapeutic and the botulism antitoxin for use in children as well as adults – despite the fact that pediatric patients were not studied due to ethical concerns during the development of these products. These achievements were made possible by the application of regulatory science. In addition, FDA also expanded approval for use of the influenza antiviral, oseltamivir, to treat children as young as 2 weeks of age. Prior to this action, oseltamivir was only approved to treat influenza in children ages 1 year and older. FDA was able to expand the approved use of oseltamivir in children younger than 1 year based on the extrapolation of data from previous studies of adults and older children, and additional supporting studies sponsored by both industry and academic researchers.

FDA has also readied medical countermeasures for potential use under its EUA authorities against a diverse array of threats including smallpox, anthrax, and pandemic influenza.¹ Furthermore, FDA issued EUAs for diagnostic tests for the avian influenza A (H7N9) virus and Middle East Respiratory Syndrome coronavirus (MERS-CoV) to facilitate preparedness for these emerging biological threats.

In the area of pandemic influenza preparedness, FDA recently approved several seasonal influenza vaccines, which helps increase and sustain pandemic influenza vaccine production capacity, including: the first seasonal influenza vaccine licensed in the U.S. produced using modern cell culture techniques, which helps to ensure a faster manufacturing startup; and the first seasonal influenza vaccine made through recombinant DNA technology, which speeds vaccine production. FDA also approved the first adjuvanted influenza vaccine for use in people 18 years of age and older who are at increased risk of exposure to the avian influenza H5N1 virus subtype contained in the vaccine. This vaccine is not for commercial distribution, but will be part of the national stockpile in the event it is needed. Furthermore, FDA has collaborated closely with BARDA, the National Institute of Allergy and Infectious Diseases (NIAID), and Centers for Disease Control and Prevention (CDC) on developing avian influenza H7N9 virus vaccine candidates.

FDA has also established a broad and robust portfolio of cutting-edge research under MCMi's Regulatory Science Program. A few examples of ongoing projects include: supporting the Wyss Institute for Biologically Inspired Engineering at Harvard University as it develops organ-on-chips models to assess radiation damage in lung, gut, and bone marrow, and then using these models to test candidate medical countermeasures; assessing the feasibility of using electronic health record systems to conduct near real-time monitoring of health outcomes, including clinical benefit or serious or unexpected adverse events associated with medical countermeasures used during public health emergencies; collaborating with the Defense Threat Reduction Agency and the National Center for Biotechnology Information to establish a publicly-available genomic sequence reference database that will be critical to developers seeking to validate their candidate multiplex *in vitro* diagnostic tests that could be used to diagnose multiple pathogens simultaneously; and

¹ To facilitate the issuance of EUAs, FDA has developed a pre-EUA submission process. FDA works with product sponsors or government agencies, such as CDC and DoD, to develop pre-EUA packages that will form the basis of an EUA request and decision when circumstances justify. Pre-EUA packages contain data and information about the safety and efficacy of the product, its intended use under an EUA, and information about the potential emergency situation that might unfold.

examining the scientific basis for the instability of the protective antigen that have hindered efforts to develop next-generation anthrax vaccines and using protein engineering to stabilize the antigen.

Details of these and other FDA successes across the full range of FDA activities within the medical countermeasure mission space appear in our annual MCMi program updates.²

Coordination with the Enterprise

FDA coordinates and collaborates extensively with Enterprise partners to foster the development and availability of medical countermeasures. FDA provides subject matter expertise and technical assistance to numerous standing Enterprise committees and working groups that develop medical countermeasure requirements, plans, priorities, and policies and that conduct program oversight and integration across the full spectrum of activities associated with medical countermeasures: threat assessments, requirements setting, product development, and procurement, stockpiling, and use.

As FDA implements its MCMi, we also engage with our Enterprise partners to ensure programmatic alignment and the most efficient use of resources. For example, FDA established a Steering Committee for Advancing MCMi Regulatory Science. This committee—which includes representatives from NIAID, the CDC, BARDA, and DoD—evaluates MCMi Regulatory Science Program research proposals for feasibility and scientific and technical merit to ensure that the MCMi Regulatory Science Program is appropriately targeted and coordinated with U.S. government medical countermeasure priorities.

Conclusion

Developing medical countermeasure is highly complex. Close cooperation and collaboration, within FDA and with Enterprise partners, is essential, and without this cooperation, the progress to address this public health challenge would be very limited. The deep engagement that is evident among the agencies represented here today is an example of public health synergy at its best. FDA is fully committed to continuing to work closely with Enterprise partners and product developers to support the development of promising medical countermeasures and to facilitating their ready availability should they be needed to respond to a public health emergency.

Thank you and I am happy to answer your questions.

² FDA's Annual MCMi Program Updates are available at <http://www.fda.gov/EmergencyPreparedness/MedicalCountermeasures/ucm270744.htm>

PANDEMICS

Mr. KINGSTON. Thank you.

Dr. Robinson, you mentioned complacency, and I do worry about that myself. I was at the CDC about 2 weeks ago and was making a statement about the lack of knowledge the average person has about pandemics in the past, particularly 1918, that area of time. And I don't know if you know any of those numbers off the top of your head, but what I would like is if you could give the committee members a memo, just a kind of a follow-up and just say, you know, past pandemics.

And nothing really elaborate, but just the years, say, 1914 to 1920, the number of deaths, maybe the countries in which it was concentrated, and the cause. Because I fear that the average school child in America, while you learn about World War I, World War II, and so forth, you don't learn about the pandemics, which cost more lives.

And I don't know if you know any of those numbers off the top of your head and want to respond on that, but I think it is important for people to understand that these threats have been out there, and the threat of them is lower than it used to be, but it is still there. Do you want to—

Mr. ROBINSON. Congressman Kingston, I would be happy to give you a memo. But in a nutshell, in 1918, the United States estimates as low as 500,000 to 1 million people in the United States lost their lives to the H1N1 1918 flu. Globally, it was tens of millions.

Mr. KINGSTON. Yes.

Mr. ROBINSON. That was more deaths than were experienced during World War I.

In 1957, we saw tens of thousands of individuals die in this country with the H2N2. H3N2, in 1968, it killed tens of thousands of people here, more than what we would normally see in our usual outbreaks every year. And then, in 2009, the H1N1 pandemic, we saw levels that were especially severe in children and pregnant women and other special populations at levels that were above what we see in our normal influenza.

There are years, in fact, where there is not a pandemic, but there are severe influenza attacks and outbreaks. And that the constant vigilance of using vaccines and having antiviral drug therapies there are a way to assuage the mortality that is associated with these. But we will be happy to give you greater details.

[The information follows:]

Information on Past Pandemics

Influenza pandemics have been recognized since 1889 with four occurring between 1918 and 2009 causing significant mortality in the millions of lives worldwide and disruption of society. In 1918 the H1N1 pandemic occurred during World War I and led to approximately 20% to 40% of the worldwide population becoming ill, causing 10 – 50 million deaths globally (675,000 deaths in the U.S.) especially in young adults; more flu deaths than war casualties in Europe and spread more rapidly than any disease in recorded history.¹² In 1957 the Asian flu pandemic (H2N2) emerged causing 70,000 deaths in the U.S. especially the elderly and 1-2 million deaths worldwide.¹ In 1968 the Hong Kong flu pandemic (H3N2) caused 34,000 deaths in the U.S. and 700,000 deaths worldwide.¹ Most recently the H1N1 influenza pandemic of 2009 saw 9,000 – 18,000 deaths in the U.S. despite mass vaccination of 80 million persons and up to 575,000 deaths worldwide.³

¹ Pandemic flu history, <http://www.flu.gov/pandemic/history/>

² Worobey, M., Han, G-Z., and Rambaut, A. 2014. Genesis and pathogenesis of the 1918 pandemic H1N1 influenza A virus. PNAS, USA.

³ Dawood, D.S., et al. 2012. Estimated global mortality associated with the first 12 months of 2009 pandemic influenza A H1N1 virus circulation: a modelling study. Lancet 12:687-695.

Mr. KINGSTON. I think it would be very helpful because it is astounding the numbers, and it is astounding that we just don't know that—you know, the average person just doesn't know about this.

The second part to this in the complacency, and Ms. Lee and I have visited PEPFAR clinics in Africa. But I think one of the stories we are not telling is how the AIDS epidemic in Africa has been greatly reduced and the cost of treatment per person has gone down as the effectiveness of it has gone up, compared to where we would have been 20 years ago. And I don't think that people understand how much stride has been made in that.

And then one thing I am always talking about as well is the success we have had in polio. And I love to go to senior citizens homes and ask them how many are familiar with polio. And every single hand is raised. And then I tell them if I go to college campuses and ask that same question, maybe 10 percent of the kids have ever heard of polio.

And you know, it is one of those success stories that we are not telling. And not that that is directly related to your work, but it does fit into the bigger picture.

And so, and my time is about expired. I don't know if you need to respond to that one way or the other. But—

Mr. ROBINSON. We will be happy to provide in a written record response to that.

[The information follows:]

COMPLACENCY ABOUT PUBLIC HEALTH EMERGENCY PREPAREDNESS

Complacency about the need for public health emergency preparedness against man-made and major natural events is a major national concern. Historically our country responds very well after an event has occurred, but prevention has not always been a strong feature of American public health as state and local health department capacity eroded and vaccination acceptance has been questioned. Despite clarion calls from international infectious disease experts, the vaccine industry has struggled over the past twenty years to successfully address emerging infectious diseases without USG assistance. This is corroborated by the lack of progress towards development of new vaccines and antiviral drugs for emerging infectious diseases such as coronaviruses (CoV) that are here today. Despite SARS-CoV outbreaks in 2003, there are no vaccines and antiviral drugs in advanced development for the ongoing MERS-CoV outbreak in the Middle East and elsewhere. The Amerithrax anthrax cases in 2001, serve as an example that we must stay prepared for man-made biothreats. Medical countermeasures are one of the national insurance policies that we must not allow to lapse, as these threats, whether man-made or natural, do occur.

NATURAL VS. MANMADE THREATS

Mr. KINGSTON. Okay. Ms. DeLauro.

Ms. DELAURO. Thank you very much, Mr. Chairman.

And I never got to know my grandfather. He died in 1918 at age 34, leaving 6 children behind, and died within about 4 days. But have seen the photographs of him, and an enormously, 6 foot, handsome man with—you know, strapping man that was just, you know, in 4 days was gone because of the pandemic.

Programs we are discussing today came out of September 11th, focused primarily on another terrorist attack, whether it was chemical, biological, radiological, nuclear weapons. Twelve and a half years later, that the investment and the time to develop the effective countermeasures is significant.

We also have threats that are evolving, naturally occurring. And so, that there has been, as I understand it from reading, in the biomedical community alarms raised because the view is that there is a heavier focus on the—that the heavier focus should be on emerging infectious diseases, growing problem of antibiotic-resistant bacteria.

So it is actually a two-part question. Given fiscal constraints, how do we go about setting the priorities between manmade threats, naturally occurring threats?

The Department of Homeland Security, do they take precedence with regard to a manmade threat? Does BARDA prioritize its support based on the likelihood and severity of a potential threat, regardless of whether or not it is manmade or naturally occurring?

And how do you all determine the balance? Where is that balance piece? And that is also in terms of the resource allocation between these two categories of need.

Let me just add to that, if I can, how often does PHEMCE re-evaluate existing threats and the funding priorities that match those threats? Do you have the nimbleness to, if you move to look at something, if something was determined in the next 12 months that was an infectious disease, drug-resistant bacteria, greater threat than a terrorist attack, how long would that transformation in BARDA, that pipeline take to move? And how prepared are we right now to address the most likely threats to emerge in 2014?

So let us talk about the balance piece and then these evaluation, continuing evaluation pieces.

Mr. KORCH. Thank you for the multipart question. There is a lot to unpackage there.

Ms. DELAURO. Right.

Mr. KORCH. With regard to identification of what threats we prioritize right now versus what threats we might see in the future, we actually in the PHEMCE, in our strategy and implementation plan, address this. For the first part of it—

Ms. DELAURO. The manmade and—

Mr. KORCH. Yes. For the first part—

Ms. DELAURO. Okay.

Mr. KORCH [continuing]. The manmade or the deliberate threats, this is a function of an evaluation that we work with the Department of Homeland Security and the material threat assessments and material threat determinations. And from that, we can project, while with BARDA's modeling capability, what is going to be the public health impact, which then informs what kind of medical countermeasures and how we do it. And that then sets the stage for the investments that we make currently in manmade.

Now on the aspects of—

Ms. DELAURO. And natural? Right.

Mr. KORCH [continuing]. Naturally occurring or emerging threats, one of the things that we look for in this program, because emerging threats is part of our mandate, not so much on the dengues and those types of problems of the world. But what we are looking at right now—and Robin alluded to this a little bit, as did Mike—is where can we develop medical countermeasures that have either platform approaches, things that we are working on right

now in biodefense, that can be almost instantly applied to other diseases that emerge.

Ms. DELAURO. This is naturally occurring?

Mr. KORCH. To naturally occurring diseases that are emerging. So different types of vaccines, other kinds of antivirals or antimicrobial. Robin can provide more information on the extensive program that is being developed right now on looking at antimicrobial resistance, new products to address the gram negative bacterial infections, naturally occurring—

Ms. DELAURO. Resource allocation, because my time is going to—they are going to—

Mr. KORCH. Okay. So let me move—

Ms. DELAURO. Resource allocation, how do you make this—you know, make the distinction and the reevaluation process involved in this?

Mr. ROBINSON. So annually within BARDA, and certainly within ASPR and then the entire PHEMCE—

Ms. DELAURO. Right.

Mr. ROBINSON [continuing]. We reevaluate what those priorities are and what the funding allocations are. So we are constantly doing that. As each individual medical countermeasure comes up for acquisition to the Project BioShield and to give to the SNS, we go through a process that goes to our senior leaders who actually say does this meet our strategy going forward?

Ms. DELAURO. I will pick up afterward. Thank you.

Mr. FLEISCHMANN. Thank you, Mr. Chairman.

And I want to thank each and every one of you all for being here today. This is not only insightful to me, as a Member of Congress and on the committee, but really it is educational and it helps me do my job. So thank you in advance.

5-YEAR PLAN

The first question I am going to ask, I will open up to ask if each and every one of you would like to respond. The second one I definitely would like each and every one of you all to respond to.

There has been a lot of discussion about the release of your 5-year plan as required by the PAHPRA. We understand that certain information that will be included in the plan cannot be released publicly for security reasons. When do you plan to release a public version of the 5-year plan?

Mr. KORCH. The plan itself will be coming to Congress hopefully by the end of March. That is our intended timeframe to address the issues, the concerns. The plan will be made available to Congress. The thought about publication to the public itself remains in consideration, but certainly providing the 5-year estimate of budget to Congress and to the administration is a big priority that we have right now.

Mr. FLEISCHMANN. Thank you.

ZOONOTIC DISEASE

My second question is directed to each and every one of you all, please. As each and every one of you all have noticed how quickly zoonotic diseases can spread once they are transmitted from animals to humans, could you please share with the subcommittee

how each of you all coordinate with other offices within the CDC and NIH, for example, and with the USDA, DHS, DoD to leverage their research activities to prevent duplication among Federal agencies and to make sure that you are prepared to anticipate and respond to threats to human health that originate in animal agriculture?

Mr. KORCH. The PHEMCE itself is constructed to have a dialogue on a regular basis with all the partner agencies that you have just described. Biweekly we meet on the problems that we face. As new problems emerge, the senior leadership of each of our organizations assesses whether it falls into the lane of the PHEMCE, per se, or happens to fall in a different part of the HHS structure.

So PHEMCE itself deals with a number of the diseases that we have described this morning, but not all diseases. Certainly on some of the agricultural ones, USDA would be the lead for that. And they would come to us, and they would bring to the table issues that we may have that we can provide solutions to or interact with, depending on the disease itself.

And I will turn this over to my other colleagues.

Mr. FLEISCHMANN. Thank you.

Mr. ROBINSON. So let me give you an example where this actually happened last year with H7N9 avian influenza outbreaks in China, which have the potential to become a pandemic. They can spread worldwide.

Unfortunately, we do have experience, and our great colleagues at CDC were able to instantly detect this. In working with our colleagues at FDA and the NIH and CDC, we were able to make vaccines seed strains in record time, using new technologies. And then actually getting those vaccines made last spring and last summer and starting them in testing with the vaccine manufacturers and with our colleagues over at NIH to do those.

And we actually have now a stockpile of H7N9 vaccine that is here in the United States, and the United States is prepared if that were to become a pandemic, with a first strike capabilities. But that is what we normally do.

We have worked together from H1N1 and other events now to H7N9 and SARS and MERS coronavirus are other examples of where we have worked together.

Mr. FLEISCHMANN. Thank you.

Mr. BUREL. I would concur with Dr. Robinson's statements. We work closely across our agency and across all of these operating divisions of HHS on a routine basis, and I think we have good connectivity to understand how to pass this information and do the right things with it for the American public.

Dr. BORIO. In addition to being members of the PHEMCE, our steering committee at FDA that prioritizes our regulatory science research is staffed by the PHEMCE to make sure that we have full alignment with their priorities. And last year, FDA also joined the NICBR, the National Interagency Confederation for Biological Research, located at Fort Detrick, which allows also to coordinate our research portfolio and, importantly, identify potential areas of synergy and cooperation to make some of the ongoing research even more useful for regulatory decision-making at the FDA.

Dr. KURILLA. The potential for zoonotic diseases to really create public health problems is something that NIH has recognized for a very long time, and we work with a wide array of partners who have available assets in terms of surveillance and other types of capabilities that they can offer in terms of being able to get the most productive results from those research.

But it is largely agent specific in terms of who we work with.

Mr. KINGSTON. Thank you.

Ms. LEE. Okay. Thank you very much.

I want to thank you, Chairman Kingston and Ranking Member, for this very important hearing today, and thank all of our witnesses for being here. But also, more importantly, for what you are doing each and every day with, again, minimal resources, quite frankly.

In my district, the Lawrence Berkeley National Lab receives BARDA funding, and they work with this funding on a medical countermeasure, and we are really pleased with that partnership. I guess I wanted to ask a few questions first with regard to partnerships with research universities as it relates to research on infectious diseases and treatments.

PARTNERSHIPS WITH MINORITY-SERVING INSTITUTIONS

How are you—do you prioritize or do you work with minority-serving institutions to develop a pipeline for diverse investigators, such as with historically black colleges and universities, Hispanic-serving universities?

Dr. KURILLA. So NIAID research funds are allocated and distributed on a peer-reviewed system. There are a variety of funding mechanisms to avoid everyone piling into the same queue, and there are some that specifically address those types of issues that you have highlighted.

And so, there are funding opportunities for those specific entities that are available, still covered under peer-reviewed system and awarded in a meritorious—

Ms. LEE. Okay. Available, but I would like to see a report as it relates to minority-serving institutions, how they fare. Are they receiving any of the funds for this very important work?

Dr. KURILLA. I think NIH would be pleased to get back with you with a written report on that.

[The information follows:]

NIAID FUNDING TO MINORITY-SERVING INSTITUTIONS

The National Institute of Allergy and Infectious Diseases (NIAID) conducts and supports basic and applied research to better understand, treat, and ultimately prevent infectious, immunologic, and allergic diseases. In fiscal year 2013, NIAID provided approximately \$162 million in research funding to minority-serving institutions in support of this mission.

Ms. LEE. Okay. Really appreciate that.

Dr. Robinson.

Mr. ROBINSON. BARDA has Small Business Administration goals, including minority goals, and which we have surpassed every year. And we would be happy to provide you.

Ms. LEE. You will give us that. Okay.

[The information follows:]

ASPR SMALL BUSINESS GOALS

ASPR's small business goal in FY2013 was 15%, and 36% was achieved. In FY2014 ASPR's small business goal is 15%, and we expect to exceed that goal again.

FUNDING FOR HOSPITAL PREPAREDNESS PROGRAMS

Ms. LEE. Also I have heard from hospitals in California that there is really a need for more sustained funding for coordination with hospital preparedness programs as it relates to sort of an integrated training with local and Federal responders. How is this coordination taking place, and do we need to do more?

Mr. KORCH. Well, certainly, we have granting mechanisms. The Hospital Preparedness Program is one of our main mechanisms for providing State, local, tribal, and territorial public health and healthcare coalitions with opportunities just for those types of needs.

We make do at this point in time with the funds that have been provided. We have seen a much more robust response in development of healthcare coalitions coming together, to offer community resilience with the funds that are provided.

The other mechanism, of course, is the PHEP grants, and I will turn over to Greg Burel to describe a little bit more of how those dollars also make it into the community for these needs.

Mr. BUREL. Thank you, George.

The Public Health Emergency Preparedness, or PHEP, Cooperative Agreement funds are those funds that our State and local public health partners use to fund their preparedness activities, whether that be planning, exercising, training, participating with hospital consortia or others in other exercises. And we send those out through our Division of State and Local Readiness, which is part of the office that also contains the Strategic National Stockpile, so that we can work closely with them and make sure that people are prepared to use what we can give them.

Ms. LEE. Okay. So if we find hospitals that aren't aware of this, can we let you know? Because they are calling and asking us for this kind of information.

Mr. BUREL. We could certainly carry that information back to the right place, ma'am.

Ms. LEE. Okay. Great.

Mr. KORCH. And the same thing with us, we will do with ASPR, with our Office of Emergency Management. We do participate in all the 10 FEMA regions. We have officers in each of the regions to coordinate these programs.

Ms. LEE. Okay. Great. And finally, let me just ask you. I held a couple years ago—well, a few years ago, a forum with regard to emergency preparedness for vulnerable populations—senior citizens, those living in nursing homes, assisted living, low-income individuals, the disabled.

VULNERABLE POPULATIONS

How are we—it was very—not very robust at that point. Are we coordinating and looking at vulnerable populations and developing strategies in the case of an unfortunate attack?

Mr. KORCH. I know that the Assistant Secretary for Preparedness Response has made a top priority in making sure that these

other organizations, which we consider still part of the healthcare community in any State and locale, they form a very important aspect of medical treatment, of being able to respond to the needs of citizens.

So those at-risk populations are part of the—of how the organization, ASPR, looks to improve coordination and capability. They certainly have over the last several years become much more integrated in our—and the PHEMCE itself, in terms of the needs of special populations, has it as one of our major goals in the strategy that we have put together.

Ms. LEE. Okay. Thank you, Mr. Chairman.

Mr. JOYCE. Thank you, Mr. Chairman.

MULTIYEAR BUDGET

Good morning. Realizing, for the panel now, the fact that President Obama's budget has not been submitted yet. So you don't have specific numbers. But did the budget process allow for the professional judgment requirement that you have identified in your multiyear planning document?

Mr. KORCH. We are using professional judgment information to prepare the 5-year program. So the professional judgment, of course, operates against the as-of-yet determined amount that is being forwarded by the administration. That, plus other information that each of the agencies has with regard to what it projects its needs are, even beyond that going into the 5 years, will constitute the information.

Some of it, present year, obviously, is known. Budget projections for the future are still estimate, as you say. But, yes, we are accounting for that information as we develop these multiyear budgets.

Mr. JOYCE. What steps, if any, do you think would be required to better align your needs with the presidential budget process?

Mr. KORCH. Well, certainly we work with our Office of Finance, the ASFR office. We work with the OMB offices. We work also with the staffs here, with the administration, to identify what we project as needs. And then, based on the prioritizations that happen within the department and the administration, we adjust appropriately to the level that resources become available.

So our estimates are ones of what we project as need to sustain the program, and then, hopefully, those are translated into the reality of the funding.

Robin, do you want to have any more?

Mr. ROBINSON. Historically, BARDA has actually been doing multiyear budgeting for a number of years because we knew that in fiscal year 2014, as Congresswoman DeLauro pointed out, we were about to go into the abyss because we no longer were going to be able to tap supplemental and advance appropriations, and we were moving, hopefully, because we were a mature organization to be considered for annual appropriations. That created a serious problem for us. It created a serious problem for you and the administration.

As we have gone forward, we presented what the outlook would look to be for the next 5 years and have done that routinely over the last several years in coordination with the CDC, FDA, and the

NIH. And who is going to be doing what and what do we actually need to fill the gaps, and then what can we possibly do with budget austerity?

MEASURING SUCCESS

Mr. JOYCE. Now Congress has required that PHEMCE evaluate the progress of all activities from research through stockpile distribution and utilization. How are you measuring success across PHEMCE?

Mr. KORCH. We look at success in a number of different ways, one of which certainly is to what extent are we able to move products through the pipeline that you have heard described, which is a very expensive undertaking, into Strategic National Stockpile.

So approval of a drug is a major success for us. Having a drug available under emergency use authorization for the need when it arises. Fostering a strong, sustainable base with our industrial partners is a major goal. Being able to be sure that when the drugs are in the Strategic National Stockpile that we are working with our State and local counterparts, public health counterparts, to be sure that those are distributed and delivered appropriately.

We have metrics, and we have processes on all of those fronts, from the industrial partners to the stakeholders at State and local and all the way through our partners here. So we do look at a number of things that we think of as achievements and use as metrics.

We also have an implementation plan with a number of tasks that have been outlined. Accomplishment of those tasks, in turn, translates to an effective PHEMCE on the whole.

Mr. JOYCE. Is there a way of improving that process as we move forward?

Mr. KORCH. Well, everything, all processes could stand improvement. And what we have seen over the number of years that we have been operating has been an increase in if you call it the bureaucratic underpinnings of this, better communication across all the members here, the ability to track our portfolios much more effectively, to look at where the challenges, the roadblocks are and to address those immediately.

We are putting in practice and play mechanisms such as portfolio tracking tools. BARDA and the CDC have very effective mechanisms for looking at whether the resources that are being used are being used in the most effective fashion. So a wide variety of metrics and mechanisms to track our effectiveness.

Mr. JOYCE. Great. Thank you, Doctor.

Mr. KINGSTON. Ms. Roybal-Allard.

Ms. ROYBAL-ALLARD. Thank you very much for being here.

PREPAREDNESS AND RESPONSE FOR AT-RISK POPULATIONS

My question has to do with preparedness and response as it pertains to at-risk populations. And Dr. Burel, limited English-proficient communities could be particularly vulnerable during a pandemic, and the reasons include a lack of insurance or access to regular medical care, lack of trust in countermeasures distributed by the Government, and lower levels of health literacy.

So this raises the concern that these populations could be left without adequate information and support during an emergency. I have three questions with that regard.

What is the CDC and its grantees doing to help ensure an effective distribution of medical countermeasures to limited English-proficient populations before and during an emergency? What models are being created that can be adapted to meet the specific needs of these and other high-risk communities?

And since many underserved, limited English-proficient populations such as those in my district receive their primary care in community health centers, what incentives to you have to encourage your grantees to work with community centers in developing their medical countermeasure distribution plans in the event of a pandemic or other biological emergency?

Mr. BUREL. Thank you for your question, ma'am.

We have done work in this area in a number of different ways over the years. Some of this is focused particularly in pandemic influenza material that we have available in a number of different languages at this time.

We have also engaged translation services to help us prepare material in advance of threats that we anticipate being prepared for so that we can distribute them in a number of languages that we know would be needed. Many of these are specific to localities, and we have also worked with States to make sure that they are prepared to do the type of messaging to provide the type of information in the appropriate language in their communities.

Some of the models that we have done to try to meet some of these specific needs that we continue to try to work with that show promise are we have personnel who work directly with our State and local partners and then with private sector business, private sector voluntary organizations who have a reach to these types of populations. And we encourage our State and local partners to continue to engage those organizations, as we do as well at a national level.

Finally, in the community health centers, we encourage our State and local partners, our grantees to work with community health centers where in their plans for distribution and dispensing of medical countermeasures that is the best place for them to do that. And these plans always make use of, I believe, the best cases in the particular locality and State to reach the affected populations.

Ms. ROYBAL-ALLARD. And do you feel confident that that is working? I mean, you use the word you "encourage" them to do this. I know you can't require them. But are you finding that, in fact, these efforts are being effective and that there is, you know, movement and there will be success in the event that we have to, unfortunately, take action in these communities?

Mr. BUREL. We have seen good work in some of these areas. We have heard stories from our State and local partners that are indicative to us that there are plans and programs in place and work being done that is effective. Anything can be made better all the time, and we continue to try to find ways to innovate and improve our capability to deliver these types of medications and services to all of these populations.

Ms. ROYBAL-ALLARD. Okay. I would appreciate, if possible, if maybe you could share with my office or the committee maybe some areas where you think even we, as Members of Congress, can be more helpful in encouraging either within our local communities or within our State legislatures to take more advantage of some of the things that you are proposing.

Mr. BUREL. We would be happy to share that, and thank you so much for that support.

Ms. ROYBAL-ALLARD. Thank you.

[The information follows:]

CDC Efforts to Ensure Effective Distribution of MCMs During an Emergency to Persons with Limited English Proficiency and At-risk Persons.

The initial response to public health threats is local. That is why CDC developed the *Public Health Preparedness Capabilities: National Standards for State and Local Planning*. Through the Public Health Emergency Preparedness (PHEP) cooperative agreement and through consultation with state and local public health departments, CDC works to ensure that health departments across the nation are prepared to respond to all hazards.

Capability 4: Emergency Public Information and Warning focuses on the PHEP awardee's ability to communicate the appropriate public health information to the public in the event of a threat. Included in this section is guidance on developing communication plans, resources on how to develop communications for culturally diverse populations, and resources for developing communications for populations with limited English proficiency, low literacy, visual or hearing impairments and other higher vulnerability populations.

Each year, PHEP awardees must demonstrate their ability to develop, coordinate, and disseminate risk communication messaging to the public. The awardee must have demonstrated the capability during an exercise or real incident.

Because the initial response to public health threats is local, CDC relies on local health officials' knowledge of their populations to best address the unique needs of their communities. To assist with these efforts, CDC field staff work side-by-side with local health officials to meet the needs of the community. For example, CDC has nine field staff working within California with one assigned specifically to Los Angeles County.

At the national level, CDC provides public health information through multiple venues (e.g. web, social media, press) to disseminate information to the public. In addition, CDC maintains close contact with elected officials to keep them informed of public health threats that are impacting their constituents.

Examples of how CDC and PHEP jurisdictions have ensured multi-lingual communications follow.

- Los Angeles County's Department of Public Health has a preparedness website (www.bereadyla.org) that includes preparedness brochures translated into Spanish, Korean, and Chinese.
- CDC's guidance for preparedness Capability 4: Emergency Public Information and Warning, includes resources to develop materials for culturally diverse communities, populations with limited language proficiency, low literacy, and for those visually or hearing impaired. Please see page 43 in the linked PDF: <http://www.cdc.gov/phpr/capabilities/capability4.pdf>.

Ms. ROYBAL-ALLARD. Do I have time for one more or not?
Mr. KINGSTON. Yes.

VACCINE HESITANCY

Ms. ROYBAL-ALLARD. Okay. This is for Dr. Korch. Over the past few years, we have seen a resurgence in vaccine preventable diseases, such as whooping cough and measles. And to some degree, it is likely tied to a growing trend among parents and individuals who are opting out of these recommended vaccines.

Last week, the CDC reported that only 34 percent of adults age 18 to 64 got the seasonal flu vaccine, and requests for exemptions from school vaccinations are also increasing in this country. And this trend may reflect a growing lack of vaccination confidence in the United States.

Are you concerned that vaccine hesitancy will be a problem during a crisis requiring the immunization of segments of whole populations of a community, especially if it involves the use of a new vaccine or a product? And if so, what is ASPR doing to address the lack of confidence in immunizations before there is a pandemic or other health crises?

Mr. KINGSTON. And Dr. Korch, we will probably have to take that for the record. But I am interesting in hearing the answer to that and may just ask you on my time the answer to it.

Mr. KORCH. Very well. Thank you.

Mr. KINGSTON. And Mr. Stewart? Having edged out Mrs. Roby by about 3 seconds—

Mr. STEWART. I pushed her down when we were coming through the hall. [Laughter.]

Mr. KINGSTON. You did. You held her up in the hall.

Mr. STEWART. All right. Thank you.

Chairman Kingston and Ranking Member DeLauro, we look forward to working with you. I appreciate your welcome. I want you to know I am happy to get your coffee. I was in the Air Force for many years, and what we did to new pilots was way, way worse than that. [Laughter.]

REP. STEWART STATEMENT

Mr. STEWART. So to the members of the panel, thank you. It is a distinguished panel, and it is one of those panels and, frankly, some of your comments make me wonder, you know, whether I and the American people are kind of smart enough to keep up with you on some of these things. And it is a little bit frustrating for you, I think, and maybe for us as well, because 3 and 5 minutes just isn't enough to really dive into these questions at all.

I would like to come back and maybe in a second round talk about some of the issues with funding and resources and whether they are adequate or, you know, how we might do a little better job of that. But I would like to keep a more broad picture, if we could, kind of a bigger picture.

Dr. Robinson, I am going to talk or mention quickly something that you said and that Chairman Kingston mentioned as well, and that is the problem we have with complacency. I don't know very many Americans who have ever heard of PHEMCE and could tell

us what it is or give us any idea, you know, of the important work that you are doing. And this panel is an opportunity to do that, I think.

You know, we had conversations about SARS, about bird flu, about other things that, thank heaven, didn't turn out the way that we feared that they might. And I worry that that gives people maybe a false sense of complacency, thinking that, you know, there are always people who are going to take care of these things. That, you know, we will see a couple of news stories about it, but it will always be in China or somewhere else, and it is not something that is going to affect us as much or very deeply.

STATEMENT TO THE AMERICAN PEOPLE

So very quickly, with one specific question, and then I want to come back to the rest of the panel and ask you to be thinking about if you had 30 seconds to address the American people, which I am giving you now, what is the one thing you would say to them? What would you want them to know about the work that you are doing?

FUNDING CONCERNS

But before we do that, Dr. Robinson, I know that the work you do with BARDA, you are always measuring threats, both manmade and natural occurring threats. You are prioritizing those, I am sure. You are trying to attach resources to those individual threats.

But at the end of the day, you run out of money. You just don't have all of the resources that you need. No Government agency does. And can you tell me quickly, are there—is there a long list of concerns that you have that you don't have the resources to address?

Mr. ROBINSON. As we said earlier, development of pharmaceutical products, medical countermeasures is risky, lengthy, and expensive. And what we do in advanced development is the most expensive portion of the entire enterprise—the clinical studies, the commercialization of the manufacturing process. So if we had more funds, there would be ability to do more clinical studies and to actually have facilities that could make these products.

We have taken the dollars that we have been given by the taxpayers to expand our response and preparedness capabilities and have actually used those, both in H1N1 pandemic and also now in the H7N9 outbreaks last year. So they are paying off already, but we are looking at ways that we can actually streamline and save the taxpayers more money by providing more assistance with risk mitigation measures that decrease the amount of failure with this whole process.

Mr. STEWART. Well, and the challenge you have—and then if I could move on to the other members. I mean, we live in a time when no agency has the resources that they would like and never will, and that is going to be a growing challenge in the future for every agency.

And again, I would like to come back to you maybe at some future point to get a feel for how comfortable you are with where we

are and how you are going to meet that challenge because I just think that is something that we have to recognize.

STATEMENT TO THE AMERICAN PEOPLE

To the other members of the panel, again, if you had just a few seconds, a few sentences, what would you want the American people to know about what you do and what they can do to be more aware and more prepared?

And Doctor, yes, if we could?

Mr. KORCH. Well, in 30 seconds, I think what we would want to tell the American people is that this is the organization and this is the group of people that are anticipating the nature of both the deliberate and the naturally occurring threats. And that as your watchdogs, we are formulating the approaches, the needs, and the capability to bring to the American people those necessary medical countermeasures that we will rely on to mitigate the effects of any of these.

Look at mortality, morbidity. What can we do to reduce the risk to the U.S. population?

Mr. STEWART. Okay. Thanks. Anyone else want their 30 seconds with a concise comment?

Mr. BUREL. I think that we would like the public to know how hard public health works to keep them protected not just from these events, but from events every day, and how important that is to them. And then also how they could look at protecting themselves better in the future, and we offer many opportunities to learn that.

Mr. STEWART. All right. Thank you, Chairman.

Mr. KINGSTON. Thank you.

Mrs. Roby.

Mrs. ROBY. Thank you, Chairman and Ranking Member. It is a privilege to serve on this committee. And so, I am thrilled for the responsibility, and I look forward to working with everybody here.

INFLUENZA

Last week, the Centers for Disease Control and Prevention Director Tom Frieden provided a briefing on this year's influenza activity. And it was very helpful when he noted that the first line of defense is vaccination, but it is important to remember that some people who get vaccinated may still get sick, and we need to use our second line of defense, the antiviral drugs.

Our focus on vaccination is appropriate, but we don't talk enough about the importance of treatments for influenza, particularly among children. According to the CDC, so far this year 52 children have died from influenza, and 43 percent of children who visit the hospital related to influenza had no complicating medical factors.

Clearly, the flu takes a serious toll on our young and vulnerable. And as a mother of two small children, I find that very concerning.

I am also troubled about news reports regarding medications that were in short supply in many parts of the country. That being said, we can clearly do a better job of avoiding shortages by having the Government be more flexible on how it approaches the SNS, the Strategic National Stockpile.

So, Mr. Burel, for example, I understand that antivirals purchased for a pandemic are packaged differently than those sold commercially for seasonal flu. This complicates drawing on a reserve to ease shortages that may occur in severe seasonal flu outbreaks.

Has the Government thought of working on a system in which the SNS stockpiles antivirals with packaging that could be used for a normal flu season? And I am told in doing so that we could expedite getting medications to the people who need them while also yielding fresher medications with longer expiration dates.

Mr. BUREL. In just this year, we worked with the major manufacturer of Tamiflu to offer to them stocks of our material should they experience shortage in their supply chain. And working with them, they were able to show us that the spotted shortage they were seeing was not something that our material at that time could be helpful for.

But we believe that trying to work towards packaging that looks similar to the commercial product would be useful in those instances. The packaging that we hold was developed specifically for us, and it was specifically developed around the time we were purchasing these materials for pandemic influenza use only. But we have moved beyond that today.

INFLUENZA VACCINE SHORTAGES

Mrs. ROBY. So what is the best way to avoid shortage headlines in the future, and are more resources necessary or can this be mitigated by implementing a better distribution strategy? I mean, you can answer or anybody else that wants to weigh in, feel free.

Mr. ROBINSON. So one of the things that BARDA has been doing with our colleagues over at the FDA is there are ways that we can use our new Fill Finish Manufacturing Network that are domestic CMOs that can actually fill these drugs that are in short supply in an emergency.

And so, we are putting forward together in 2014 a plan to go forward with the FDA to be able to utilize these four different partners that we have in the private sector.

Mr. BUREL. At CDC, we continue to look for ways to work with private sector partners that do this work every day for the distribution and dispensing of these countermeasures or these medications for seasonal flu, to look at is there a place and is there the right opportunity to use some of our material to assure that shortages don't occur and that things flow through a normal supply chain, as people expect.

Mrs. ROBY. Okay. Did you want to? Go ahead.

Dr. BORIO. I would just add that FDA works very closely on this issue, as well as CDC. And during the spot shortages, for example, we sent communications out to remind pharmacists and physicians that the label of oseltamivir, Tamiflu, contained information about how to prepare pediatric formulation in the absence of suspension.

And also we sent communication out to our State and local health partners to remind them that certain stocks of Tamiflu were still good for use based on our scientific analysis and should not be discarded.

Mrs. ROBY. Okay. And speaking of suspension, real quick, I mean, how much are we supposed to have on hand for children that can't—you know, that need this in the liquid form?

Mr. BUREL. So I would ask to come back to you with the specific numbers that our current stock requires in that area.

Mrs. ROBY. Okay. Okay. That is great.

[The information follows:]

SNS REQUIREMENTS FOR ORAL SUSPENSION ANTI-VIRAL COUNTERMEASURE

The PHEMCE target goals for SNS antiviral suspension (specifically oseltamivir) holdings total 1.6 million treatment regimens; however in an effort to make the best use of taxpayer funds, CDC does not maintain oseltamivir suspension in the SNS. If suspension product was needed during an emergency, there are instructions available for compounding suspension formulation from oseltamivir capsules. To meet the needs of pediatric patients, SNS also contains pediatric strengths of oseltamivir capsules (30mg and 45mg capsules). These capsules may also be opened and put into different mediums to facilitate medication administration for children who may not be able to swallow capsules.

These solid oral dosage forms can be purchased and maintained in the SNS at significantly lower cost than suspensions, allowing CDC to maintain greater treatment capability to protect the U.S population within available funding resources.

Mrs. ROBY. I yield back.

Mr. KINGSTON. Thank you.

And we will start our second round now, and I am assuming everybody is staying for a reason.

VACCINE HESITANCY

Dr. Korch, I am going to ask you to answer Ms. Roybal-Allard's question, but I want to put this specific on it that we hear from time to time from constituents that some of the vaccines have an unintended consequence of causing autism. So there is some suspicion that these vaccines maybe aren't as tested as they should be.

COMMERCIAL INTEREST AND FDA

So I would like you to answer that. But while he is doing that, Dr. Borio, and I am going to give you a minute, and then would like to talk—I would like you to answer a question in terms of the commercial interests on FDA oversight. Drug development companies is proprietary because they have to be proprietary. They have to get approval, and they need to work with you toward some of these goals.

And I would just like to ask you a general question in terms of the balance. So you can be thinking about that while Dr. Korch is answering the question.

VACCINE HESITANCY

Mr. KORCH. Well, we certainly recognize the public perception or perception in some part of the public with regard to safety and efficacy of vaccines. Now, as you all know, vaccines, the use of vaccines in our society has largely been responsible or is a very important component of mitigation of childhood diseases.

As you mentioned earlier, polio, well known to our grandparent and parent generations, not so much known today. That is a direct result of very effective vaccines. Even across the world, the ability

almost to control now polio across the world, it has got its problems.

Now the question of the safety is extensively tested prior to the release of the product and then after the release of a product. We do postmarketing surveillance.

The literature that is expanded regarding the issue of autism, its linkage to vaccines, there has been a tremendous amount of focus in the scientific community, in the research community, to look for those linkages. My understanding is, from looking at the literature, that has largely been disproven, at least in the scientific literature that has been presented.

But the messaging around that still continues to be a major challenge. The Centers for Disease Control, the Food and Drug Administration, I think we all have responsibility, when you talk about complacency, this is the flip side of that. Complacency or not wanting to know what the issues are at hand that we face on a daily basis is one aspect. But then the effects of information that is spread in ways that when evaluated against the scientific information we have still has a corrosive effect.

We will rely upon these vaccines or some of the vaccines that we are developing, should we see an event. I think the cost-benefit ratio that people make on an individual basis changes as a function of "Am I facing an imminent threat or not?"

Those are factors that we would have to weigh on as an event happens. But certainly, our job is to make those products available, make them safe, make them effective.

Mr. KINGSTON. So you would say wholeheartedly to the moms and dads of the world get vaccinated. Get your children vaccinated.

Mr. KORCH. My kids are vaccinated.

Mr. KINGSTON. Okay. Dr. Borio.

COMMERCIAL INTEREST AND FDA

Dr. BORIO. To that end, I will just add that surveillance, safety surveillance of products in use is something that we all take very seriously and collaborate on. And H1N1 was a real important example of how product was deployed for use, and our ability and close cooperation with the CDC took a lot of safety information in near real time for over 100 million doses. It was essential to give the U.S. Government the confidence they needed to be able to communicate with parents and the population the safety profile of the vaccine.

Mr. KINGSTON. Well, let me ask you about the commercial balance and approval and that question.

Dr. BORIO. So how we prioritize—

Mr. KINGSTON. Yes.

Dr. BORIO. So FDA works with all sponsors and developers, all sponsors and developers. Clearly, this area of countermeasures is one of the most complex types of products for us to help develop and to evaluate.

When resources are very, very limited, we will prioritize the products in defensive pipeline because they do represent the best thinking of the U.S. Government with respect to priorities. Not uncommonly, however, we will also inform PHEMCE of products that we encounter during our interactions with the commercial sector

that may be very promising, that have unique features, for example, that ought to be considered.

So there is a constant exchange of information, but it is our priority to support the enterprise with these resources.

Mr. KINGSTON. Thank you.

Ms. DeLauro.

Ms. DELAURO. Thank you, Mr. Chairman. I wasn't going to go here next, but I will. I am presuming we have a third round.

PUBLIC INTEREST IN BARDA INVESTMENTS

This is a very, very interesting piece. Something that I am interested in, and I think it gets me to you, Dr. Robinson, and this is about the public interest in BARDA investments. Because BARDA provides substantial assistance, financial and otherwise, to companies for development of drugs, vaccines, and other products.

What I want to try to get at here is the public interest here, protecting that public interest and U.S. taxpayers with regard to this investment. The return, which we would agree on, is a benefit, whether there is a terrorist attack.

But what I want to know is—and I am going to send a list of questions on this. So I am going to truncate what I want to say is here is when you—how is it that we deal with the contracts under BARDA and BioShield about negotiating, how does BARDA negotiate contracts? How do we negotiate price with regard to BioShield?

Is competitive bid laid out here in any way? Are the U.S. taxpayers, just quite bluntly, are they paying for high profit margins? And what are the—what have we been paying for product development? What are we doing? It just appears that we—my understanding is that we support the facility. There is a match to do here that is then we support the technology and the development, and then we are the purchaser.

So we support that effort. So I am very interested in how these contracts and these prices are set and what we do to monitor the costs of all of this effort.

Mr. ROBINSON. Thank you, Congresswoman.

You get to the heart of it, and that is how we be good stewards of the taxpayers' dollars.

Ms. DELAURO. Amen.

Mr. ROBINSON. BARDA, we feel, is a really good steward because we do get to see the entire process from when the NIH turns over a product to us to actually then we going through advanced development to turn it over to the Strategic National Stockpile.

So we follow the Federal acquisition regulations. We use independent Government estimates to determine what is the price for like products out in the public. And we have actually done a preliminary study that actually shows that the length of time and the amount of money that the medical countermeasures that BARDA, NIH, and CDC has supported is actually much less than what we see in the private enterprise.

And in terms of about a \$1,000,000,000 to \$1,200,000,000 is what it takes for a product to go from beginning to becoming a marketed product. We are seeing anywhere from about \$300,000,000 to

\$400,000,000 for many of our medical countermeasures. How are we able to do that?

Ms. DELAURO. Do you competitively bid? Do you go to various places?

Mr. ROBINSON. We absolutely do. There has been only one instance in which we did not, and that was for the urgency of that product that we needed—

Ms. DELAURO. What was that, anthrax?

Mr. ROBINSON. That was for the smallpox antiviral drug.

Ms. DELAURO. Smallpox, okay.

Mr. ROBINSON. We have responded to that on a number of occasions on why we did that. But it was, in fact, middle of the road in its price range of commercial products that were like other antiviral drugs, in fact.

As we go forward with our core assistance program, if we can mitigate failure so that we have more successes, which from the beginning usually is around 90 percent of the products, the candidates that go through fail. We decrease that, then we actually don't have to pay for all those failures. And we are doing that through our core service—

Ms. DELAURO. Right. Because, look, we are the buyer. I mean, you know, there may be commercial aspects of this, but we are primarily the buyer.

Mr. ROBINSON. That is right.

Ms. DELAURO. We don't get the benefit of the commercial profit. The companies get that benefit. Do we put a timeframe, and are we watching the timeframe that says, okay, this is what we said you were going to do. These are the milestones, et cetera. You are not there. So, you know—

Mr. ROBINSON. Well, we—

Ms. DELAURO. Or are we saying, my God, this is the only place we have to go, and we may have an emergency, which means we just flood in more money to deal with what they said. You know, do they come in budget and on time is what we are talking about.

Mr. ROBINSON. You will probably hear grievances about BARDA being pretty tough about down-selecting. We do products that don't make it.

Ms. DELAURO. I will have a list of questions in this area, which I would like to submit to you.

Thank you.

Mr. KINGSTON. Mr. Fleischmann?

Mr. FLEISCHMANN. Thank you, Mr. Chairman.

PROJECT BIO SHIELD

And in, I guess, furthering the line of questioning from my colleague Representative Stewart, Dr. Robinson, I have a question for you, sir. The subcommittee was somewhat surprised by the budget request that was sent up by the administration last year, sir, for the Special Reserve Fund. I can imagine that there is substantially more product you can purchase than the \$250,000,000 would allow.

In an ideal world and in your best professional judgment, can you tell us how much money you could reasonably spend in fiscal year 2015 to adequately and responsibly protect the American public from the various forms of bioterrorist attack?

Mr. ROBINSON. Thank you for the question, Congressman.

In the President's budget that you will see coming forward, you will see a significant increase in the amount of funds that we will be requesting for Project BioShield and the Special Reserve Fund above what we asked for in fiscal year 2015. That will actually go towards three new products that we think that we can bring to the American public in preparedness and response.

Those would include a chemical agent antidote, a thermal burn treatment, and also a biodosimetry device in which we can actually measure the amount of radiation that someone has actually experienced after an event.

Mr. FLEISCHMANN. Thank you, sir.

STRATEGIC NATIONAL STOCKPILE

Mr. Burel, we have noticed a trend in recent years that budget requests for the Strategic National Stockpile have declined. In fiscal 2014, we appropriated \$535,000,000, an increase over fiscal 2013.

With over 80 products in the pipeline, in an ideal world and in your professional judgment, can you tell us how much money could reasonably be spent on the SNS in fiscal year 2015 to replenish existing products and purchase new products in the BARDA pipeline to adequately and responsibly protect the American public, sir?

Mr. BUREL. BARDA has done a lot of great work to make sure that we get the longest life span available from the products that they provide to us, and I think that is a real success story for us and BARDA together to have found ways to be able to get the longest life out of what they have been able to invest.

So I believe that we are prepared to invest appropriately to replace anything that needs to be replaced from that BARDA pipeline in the next year. But there is nothing specifically that we need to replace from the BARDA pipeline, I believe, until 2018.

Mr. FLEISCHMANN. Thank you, Mr. Chairman. I yield back.

Mr. KINGSTON. Ms. Roybal-Allard.

CUTS TO PUBLIC HEALTH AND PREPAREDNESS

Ms. ROYBAL-ALLARD. Dr. Burel, a recent NACHO survey reported that due to sequestration and other budget cuts at the Federal, State, and the local level, health departments have lost tens of thousands of public health jobs, and health departments are reporting cuts to their emergency preparedness programs.

As we approach the beginning of the fiscal year 2015 budget process, can you explain to this committee the impact these budget cuts and sequestration have had on our preparedness, and what impact, if any, will additional cuts to the Public Health Emergency Preparedness grants have on our ability to distribute medical countermeasures?

And what needs to be done to help communities restore their lost capacity to enable them to effectively respond to a health crisis?

Mr. BUREL. The work that our States and locals do with the funds that were provided under the PHEP Cooperative Agreement directly helped them be prepared to make use of the medical countermeasures in the Strategic National Stockpile.

We believe that they continue to find more new and creative, innovative ways to work within the funds that they have. But as availability of funds continue to erode in that area, we have to continue to work with them to provide them the right things and tools and assistance to make the best use of the medical counter-measures that we can give them and to help them plan and prepare as well.

Ms. ROYBAL-ALLARD. But directly with these lost tens of thousands of public health jobs, will you—with whatever these new measures that you are talking about, will it be sufficient to fill those gaps in the event of an emergency? Or do we need to consider perhaps investing more into these grants to get our communities better prepared?

Mr. BUREL. We will continue to look at the measure data that we have available, and we can come back to you with more information about areas in which State and local public health could benefit further.

Ms. ROYBAL-ALLARD. Okay.

Ms. DELAURO. Would the gentlelady yield for a second?

Ms. ROYBAL-ALLARD. I will yield, yes.

Ms. DELAURO. With all due respect, and I understand the response to Ms. Roybal-Allard's question. But the fact is, is that over the past decade, we have seen a real cut of about 42 percent, \$465,000,000, to CDC if you count for inflation, et cetera, for State and local agencies over this last 10 years.

That has got to have some impact on our preparedness at the State and local level. It is not doing more with less. It is doing less with less. And I think you have an obligation to let this committee know what is happening out there to our preparedness.

Ms. ROYBAL-ALLARD. That is the point, and I think that all of us, regardless of what side of the aisle we are on, really want to know what the impact is of decisions that we make. And even within the budget restraints, we want to be able to do whatever we can to make sure that our country is prepared.

So I agree. It is important that we have to have realistic answers so that we, together, can make decisions on how we could, you know, best support the needs of our countrymen. So I will just leave it at that, and hopefully, if you could give us some additional information, I am sure that all of us—in fact, one of my colleagues on the other side of the aisle was asking a similar question, wanted to get a better understanding of the consequences of what we are having to deal with.

Mr. BUREL. Thank you, ma'am, for that, and we will provide additional answers.

[The information follows:]

Rep. Roybal Allard Question: Are PHEP funds and the measures undertaken to assist the state and local health departments sufficient or should Congress “consider perhaps investing more into these grants to get our communities better prepared?”

Rep. De Lauro Question: What are the impacts/consequences of PHEP funding reductions?

CDC’s Public Health Emergency Preparedness (PHEP) cooperative agreement is the primary funding mechanism supporting state and local public health agencies in developing preparedness capabilities necessary to plan for a public health event or emergency, including medical countermeasure dispensing. Since 2002, PHEP cooperative agreements provided nearly \$9 billion to public health departments across the nation to upgrade their ability to effectively respond to a wide range of public health threats. In FY 2015, CDC’s continued support of public health departments through PHEP will focus on capability sustainment. This decrease also eliminates \$8.1 million for Academic Centers for Public Health Preparedness. CDC will continue to support research and training for public health preparedness through the research agenda of the OPHPR Science Office.

CDC has developed national standards for building and maintaining 15 public health preparedness capabilities at the state and local levels. These capabilities include public health laboratory testing, public health surveillance and epidemiological investigation, community preparedness, medical countermeasure dispensing, emergency operations coordination, emergency public information and warning, and other key capabilities that are the cornerstone of an effective response at the local level and help to promote safer, more resilient communities.

State and local public health departments use PHEP cooperative agreement funding to build and sustain these capabilities based on their jurisdictional priorities. Recent PHEP assessment data show that in the aggregate, awardees have sustained the improvements in public health capabilities.

A CDC analysis of the impact of PHEP funding on state and local public health preparedness capability development indicates that when PHEP funding was at historically higher levels, PHEP awardees invested a greater share of their resources into building critical preparedness capabilities that did not exist previously. As funding has decreased, awardees use more of their available funding to maintain existing capabilities.

Mr. KINGSTON. Mr. Joyce.
 Mr. JOYCE. I pass.
 Mr. KINGSTON. Mrs. Roby.
 Mrs. ROBY. Thank you.

BARDA NOT-FOR-PROFIT FUNDING

Just real quickly, Dr. Robinson, given the contributions for the not-for-profit research community to biodefense, how is your agency utilizing the resources of these type organizations to fulfill your mission?

Mr. ROBINSON. So we have a short history because we have only been in existence for about 7 years, but we have worked with a number of nonprofits. I will give you a really good example of where we have worked with PATH and the World Health Organization to actually build influenza vaccine manufacturing not only here in the United States, but more importantly because pandemics are everywhere in developing countries.

And so, we have actually worked since 2005 with WHO on this, first with starting with just a single grant in Vietnam, and then now moving towards 11 different countries and 13 different organizations in those countries to produce influenza vaccines. We started with zero doses they were able to do. Today, they can make 330 million doses.

Our goal by 2015 is 500 million doses. That relieves the burden of what we have to provide to the rest of the world as we go forward into these big events like a pandemic.

That contribution so far has been \$65,000,000 that BARDA has helped with. For every dollar we put in, there has been \$5 to \$7 in these different countries with other agencies and those countries to build this capacity.

So we look forward to more engagement with Gates and other nonprofit organizations going forward.

SPECIAL RESERVE FUND

Mrs. ROBY. Can you talk just real quickly about the 10-year guaranteed appropriation for the SRF? How that has had a positive impact, and you know, companies and institutions have been able to invest in R&D.

But now, and as I understand it—again, new member of the committee—but the shift to the annual appropriations process makes business planning more difficult. So how do you continue to incentivize the industry so that these companies stay in that space?

Mr. ROBINSON. This is really a good point, and this is sort of one of the big points of this hearing, in fact, is what are the next 5 years going to look like for companies out there that have been steadfast partners of ours in the biodefense space over the last 10 years?

So they have delivered 12 products so far. We think in the next 5 years they will deliver another 12 under Project BioShield. And how much money is that going to cost?

Well, Congress authorized \$2,800,000,000 over the next 5 years to do that. Our estimates are that we will need every single penny of that and maybe a little bit more in order for those 12 products

to be realized and to make us more prepared and better to respond going forward.

Certainly, in our annual meeting that we call BARDA Industry Day, we have indicated what those products are and the amount of funding overall that we will need for that. In our coming multiyear budget you will see, it will certainly reflect that level of funding that we will need to make that happen.

Mrs. ROBY. Thank you. I yield back.

BARDA PROCUREMENT AND CONTRACTS

Mr. KINGSTON. Dr. Robinson, in terms of your integration with the Department of Defense, I am sure you work hand-in-glove with them, but sometimes we worry about their procurement, how effective it is, if there are stumbling blocks. Do you work on the same processes and contracts, or just how does that work?

Mr. ROBINSON. So biodefense has a long history with the Department of Defense, and we and NIH and the CDC and FDA have built off of what they started prior to 9/11. Going forward, we have worked with them in some cases to actually pick up product candidates that they were no longer able to fund because of changes in their budget and also to coordinate on a daily basis not only visibility of what they are doing and what we are doing, but also then to shift over.

And we certainly have picked up a number of products from them and certainly from NIH. We are coordinating those efforts in animal studies, the types of candidates, and also with a new strategy that we have for flexible manufacturing. We at HHS have established three Centers for Innovation in Advanced Development and Manufacturing. A fourth center is being supported by the Department of Defense, and in several years, that hopefully will be constructed and able to join in this national effort to build a response network to provide these medical countermeasure products.

Mr. KINGSTON. I am going to go ahead and yield the balance of my time to Dr. Harris, just to expedite things. If you are ready to hit the ground running, you have 3 minutes.

Dr. HARRIS. Thank you very much. I hit the ground running fast.

PANDEMIC INFLUENZA AND STRATEGIC NATIONAL STOCKPILE

Thank you all for being here today, and I have a couple of questions that one is that during—you know, we have this issue of these kind of manmade versus natural emergencies. And of course, the one that came up—the only one that has really gone into effect since then, since this whole concept really is a natural one. I mean, it was the pandemic.

So during the pandemic, 2009 pandemic, there were—my understanding, there were several million, it is 12, 13 million courses of antivirals distributed in anticipation of requirement for the pandemic. And I don't know who would—I don't know if they came out of the stockpile or not, but where are they? What happened to them?

You know, the pandemic didn't pan out. I mean, so where—what happened to that asset?

Mr. BUREL. So the antivirals that were distributed from the Strategic National Stockpile, some of those were actually dispensed.

Some of those were moved into State holdings so that they could be used in the future.

To give you an exact breakdown, we would have to come back to you with those numbers.

Dr. HARRIS. So they were dispensed, but they were—when you dispensed them to the States, I take it that there are plans in the future, for instance, if let us say I don't know where the projects were that this pandemic was going to occur, but I would imagine the projection is not that it is nationwide. There would be pockets.

There are mechanisms to recall that item from those States and redistribute? I mean, that is part of the planning in the future?

Mr. BUREL. In our current planning, once we release assets to the States we don't pull those back to the stockpile. But the States do have plans in place that they can cross-level those materials in their States.

Dr. HARRIS. But through your—through the stockpile, or that is left up to their own devices?

Mr. BUREL. That is left up to the State.

Dr. HARRIS. Why?

Mr. BUREL. The State has capability internally that we have worked with them in their planning to assure that they can provide medical countermeasures that we have given them to the places where they need to dispense it.

So in some cases, States have chosen to place that into the pharmacy supply chain. In other cases, they distribute it through other means and places.

PHARMACY SUPPLY CHAIN

Dr. HARRIS. And when it goes into the pharmacy supply chain, where do the funds—I mean, are they reimbursed through the pharmacy supply chain? I mean, obviously, those get sold. Money is exchanged. Does it come back to the Federal Government? Does it stay in the States?

Mr. BUREL. Product that is provided from the Strategic National Stockpile, regardless of where it is dispensed, is not charged. In the H1N1 situation, pharmacies were allowed to charge a small administrative fee to dispense a drug, and I believe that that was set by CMS pricing. But I would have to check, sir.

BIOLOGIC THREATS

Dr. HARRIS. Okay. And when I reviewed the report, the GAO report, you know, it seems that there are a lot of potential targets/threats, goals. I mean, 255 I think was the total number, whatever. But then when you look at what has actually been procured and put in the stockpile, it seems that in terms of diseases, there are only two, two things.

I mean, there is botulinum and—there is three. There is botulinum, anthrax, smallpox. Are those the only biologic threats that we have? I mean, how many other biologic threats among those 255, you know, targets?

Mr. KORCH. Well, there are 13 material threat determinations across chem/bio/rad/nuc. Of those, we have got anthrax, smallpox, bot, as you mentioned. There is tularemia, plague, viral hemorrhagic fevers.

The products that we have procured already for the most part were those products that were most mature, could be used effectively with our Special Reserve Funds. The products that are further back in the pipeline because they are less mature, example, hemorrhagic fever viruses, for which there are at this point still mostly candidate products way back at the tech base in our research labs.

So the decision has to be made. You have got funds to spend on products that are now currently available to bring them across the finish line. That is the goal of BARDA to adjust. Certainly, we look at what are the most important threats? What do we think from our information and our working with Department of Homeland Security really require the direct attention?

The ones that you mentioned are important threats, and so, but in addition to those, of course, we are looking at what do we do for radiological and nuclear? How do we stopgap the materials there as well?

The 255 tasks that you mentioned don't necessarily relate to 255 different pathogens. They relate to—

Dr. HARRIS. No, I understand that. And will some of these—some of this will be revealed in the plan that is going to come out in the spring?

Mr. KORCH. The spring plan will identify where we are spending or what we project to spend on a threat-by-threat level, yes.

Dr. HARRIS. Okay. Thank you very much.

Thank you, Mr. Chairman.

Mr. KINGSTON. Ms. DeLauro.

Ms. DELAURO. Thank you, Mr. Chairman.

SUBCOMMITTEE ALLOCATION

Just a quick comment to Dr. Robinson. I do hope the industry partners will recognize that we have been steadfast supporters of their efforts as well. And when we are going to have to take a look at, as I said in my opening statement, if the allocation to this subcommittee does not increase substantially in the next go-around, that we will not be able—we are going to have to create a balance as to what gets funded and what doesn't.

So I think industry ought to—but they ought to be thinking about the humanitarian aspects of their participation in this effort. They certainly do get provided with a number of a lot of U.S. tax dollars in order to do what they do, and they do it very well. I don't take that away from them.

INFLUENZA VACCINE

Just a couple of questions on, just on the immunization against flu. Dr. Kurilla, I understand that NIAID is doing research on a universal influenza that is protection from all strains. Can you give us an update on the status of that research and how far are we from a human trial? And I am going to ask for a short answer. I have got a couple of others and then questions I want to ask as well.

Dr. KURILLA. Well, the development of a universal flu vaccine has been the holy grail in seasonal flu and would also address pan-

demic flu for a very long time. And we take seasonal and pandemic flu very, very seriously.

The scientific basis conceptually for the potential for a universal flu vaccine is something that has been emerging over the last several years, and there are a number of various concepts that are moving forward through preclinical. We hope in the next couple years to be able to advance for some clinical testing to test the feasibility of that potential, but we are still a ways away from definitively having solved that—

Ms. DELAURO. So we are a ways away from clinical trials, and then we are a ways away from the clinical trials until—

Dr. KURILLA. Large Scale Trials. Yes.

Ms. DELAURO. There again, in terms of the—okay. So it is a ways off here? Okay.

Dr. KURILLA. Yes. But very promising.

Ms. DELAURO. I understand. I understand. With regard to BARDA, recombinant flu vaccine technologies, Dr. Robinson, and obviously, this was drawn up from the Council of Advisers on Science and Technology as a way for us to move faster to have a better response to the next pandemic.

INFLUENZA INVESTMENTS

Efforts in that area and what other investments has BARDA made to speed up the response time in the next flu pandemic?

Mr. ROBINSON. So we have supported over the last 7 or 8 years the development of modern technologies, including cell-based and recombinant-based, that resulted in Flucelvax being licensed in 2012, and then last year in 2013, Flublok, a recombinant-based vaccine, being licensed.

We are supporting two other recombinant-based vaccines that are in late-stage development, and one of them actually went forward. It was one of the first H7N9 vaccine candidates that we had. It was tested and looked very promising for that.

And so, we, I think, have brighter horizons coming forward with recombinant and also other technologies going forward for flu vaccines and speeding it up. And we certainly had the H7N9 vaccines available quicker than we have ever had vaccines available in the past.

ANTHRAX

Ms. DELAURO. Let me ask about next-generation anthrax. Where are we with anthrax? Just someone bring us up to date.

The understanding is the existing vaccine is problematic, that we need to produce a next generation that will be safer. It is underway for years now and again spent a fair amount of money already here.

Where does the product stand in the pipeline, and when do you expect this next generation to be ready for the stockpile?

Dr. KURILLA. So in terms of anthrax, we regard the anthrax program as a mature program because there are multiple products in the stockpile to address the threat of anthrax, which includes a vaccine, which we feel is safe and effective as it exists, as well as antitoxins to deal with anthrax disease and antibiotics, which are, in fact, the primary response to anthrax.

That being said——

Ms. DELAURO. Is that the one that Department of Defense uses, the one you are speaking about now?

Dr. KURILLA. Yes. But, so with a mature product, what we look for for next generation is improved performance of that product and other product attributes that are advantageous in terms of enhancing our preparedness, as well as reducing the cost and making easier distribution and administration of those products.

And so, in the case of anthrax vaccines, we have a number of candidates that are moving forward, some of which are already into Phase II testing, that will reduce the number of doses of anthrax vaccine that would be required to produce an effective level of protection. We also are looking at very early, which is just beginning clinical testing, the possibility of an oral anthrax vaccine, which would really simplify the degree of administration.

And then we have another of next-generation recombinant vaccines, which are being looked at both in terms of product stability, that is the potential to store the vaccine at room temperature, which reduces storage, long-term storage costs, extends the shelf life, and makes distribution much easier because of elimination of the cold chain, as well as a number of alternative delivery devices that require less trained healthcare workers for administration.

Ms. DELAURO. How many years away are we from this? Just, and I am not—this is not an——

Dr. KURILLA. Well, I can't predict the success of any individual product.

Ms. DELAURO [continuing]. Adversarial question, just to get a sense of where we are.

Dr. KURILLA. Based on industry standards, a Phase II product, we are maybe looking in possibly maybe 3- to 5-year timeframe to possibly be considered for licensure. Some of the earlier ones entering clinical trials would add a few more years. After that, we are maybe in the range of 10 years or more.

Mr. ROBINSON. Mr. Chairman, may I have your indulgence just so I can amplify? BARDA actually has four recombinant candidates, and we think that by fiscal year 2018, we may actually have one that is mature enough for consideration of Project BioShield to put in the stockpile.

Ms. DELAURO. Thank you.

Chairman, I understand I can finish?

Mr. KINGSTON. Absolutely.

Ms. DELAURO. Okay. Thank you.

Mr. KINGSTON. Dr. Harris, you are finished?

Ms. DELAURO. Okay. Thank you. Thank you both very, very much.

BARDA AND NCATS

BARDA and NCATS, we created the new Center for Advanced Translational Sciences to focus on some of these issues—basic research, viable drugs, therapeutics, diagnostic tools. They seem to have many similar types of applications, the kinds of problems that you both are working on.

Just quickly, the collaboration between the two organizations and how you work with one another or how you will work with one another because it is new?

Mr. ROBINSON. I will let my colleague Dr. Kurilla follow up here. But we have worked with the NCATS with Chris Austin and now with Pam McGinnis there. And actually are shaping up going from concept to actual projects that we think that we can actually have synergy on so that we are not duplicating what we are doing.

Ms. DELAURO. Right.

Mr. ROBINSON. But actually together we can do something that neither one of us can do together, especially maybe on antimicrobial drug.

Dr. KURILLA. The other thing I would add is that NCATS does offer some special features in terms of drug screening, and we have taken advantage of that, particularly with emerging infectious diseases, that is a collaboration between NCATS and NIAID with regard to biocontainment requirements in order to conduct those screenings.

And that is going on, and that is a potential being utilized right now for the MERS-CoV and would be available for other potential BSL-3 dangerous pathogens that would require higher levels of containment.

With regard to biodefense in general and product development for infectious disease, though, NIAID has all the authority that it needs and the requisite mechanisms in order to advance our candidate products through preclinical and into early phase human testing to make them eligible for transition to BARDA. And so, we can handle those ourselves.

Ms. DELAURO. Okay. Thank you.

SHELF-LIFE EXTENSION PROGRAM

Dr. Borio, 2004, the stockpile has been a participant in shelf-life extension program, and you do the analysis to determine beyond expiration date. I understand that FDA is only authorized to provide this service to specific Federal agencies—DoD, CDC. It is not available to State and local agencies and their stockpiles of countermeasures.

State and local efforts have stockpiles—Cipro, Tamiflu, et cetera. They are expensive to maintain and to replenish. Have you explored the option of opening a self-life extension program to State and local health agencies, or is there another way that you are looking at in helping them to maintain their own stockpiles?

Dr. BORIO. So it is a fairly long answer, and I would like to provide an answer to that to the record in written form.

Ms. DELAURO. Perfect.

Dr. BORIO. But I would also like to say that PAHPRA gave us explicit authorities to allow us to extend shelf life of certain products, and we have used those authorities very frequently since PAHPRA, including just this week with intravenous saline shortage situation. For example, we were working with CDC to extend the expire dating for their products.

So we use the authority several times, and I will provide a more comprehensive response.

Ms. DELAURO. That would be good because I know it is a big concern with the State and local.
[The information follows:]

Shelf-Life Extension Program

What is SLEP?

The Shelf Life Extension Program – or SLEP – is the federal, fee-for-service program through which the labeled shelf life of certain federally stockpiled medical materiel, such as medical countermeasures held in the Strategic National Stockpile (SNS), can be extended after periodic stability testing conducted by FDA. The program, which is administered by the Department of Defense (DoD), was established in 1986 under an inter-agency agreement with DoD and FDA after it was recognized through testing that certain products remained stable beyond their labeled expiration dates when properly stored. Through expiration dating extensions, SLEP helps to defer the replacement costs of certain products in critical federal stockpiles. Under the program, DoD performs programmatic and administrative functions and FDA performs testing and evaluation of drugs for expiry dating extension. SLEP is available for federal agencies who sign a memorandum of agreement with DoD. Current participants include DoD (Army, Air Force, Navy, Marines), the SNS (managed by the Centers for Disease Control and Prevention), the Department of Veterans Affairs, the United States Postal Service, and the Bureau of Federal Prisons.

What are the Challenges to Expanding SLEP to State and Local Medical Countermeasure Stockpiles?

State and local response partners have in the past expressed interest in participating in SLEP to help them maintain their own stockpiles of medical countermeasures. However, expanding SLEP to state and local medical countermeasure stockpiles presents non-regulatory programmatic, administrative, and infrastructure challenges for the managing entities. For example, expanding SLEP would create a large increase in SLEP customers, which would require additional infrastructure, personnel, and resources to manage and monitor the program. Additionally, DoD currently performs the programmatic and administrative functions of SLEP, however expanding SLEP to state and local partners would require a reconfiguration of these duties. Thus, expanding SLEP to state and local medical countermeasure stockpiles may negatively impact the functioning of the current SLEP (especially if additional resources were not available to support the program expansion). Furthermore, expanding SLEP may not be a cost-effective solution for state and local partners due to stockpile size, the costs associated with any required testing and relabeling compared to the cost of replacing product, etc..

How Has FDA Otherwise Worked to Support State and Local Stakeholder Expiration Dating Challenges?

Before the Pandemic and All-Hazards Preparedness Reauthorization Act of 2013 – or PAHPRA— was enacted, the distribution, dispensing, or use of medical countermeasures with extended expiry dates, and any related labeling adjustments, was possible only by the exercise of FDA’s enforcement discretion or through the issuance of an Emergency Use Authorization (EUA) by FDA. Recognizing the need for improved legal clarity with respect to FDA’s ability to extend expiry dating of medical countermeasures, FDA worked with the Department of Health

and Human Services (HHS), the Administration, and Congress to enact changes to FDA's statutory authority under PAHPRA. These changes provide FDA with the explicit authority to extend the expiration dating of FDA-approved medical countermeasures stockpiled for use in public health emergencies (e.g., an anthrax attack or influenza pandemic), if the extension is supported by an appropriate scientific evaluation.

FDA has proactively used the PAHPRA expiration dating authority to respond to critical medical countermeasure expiry dating challenges at the state and local levels as well as for certain federal stockpile issues. For example, FDA used this authority to ensure that the nation's first responders and warfighters will continue to have ready access to auto-injectors used for the treatment of nerve agent poisoning after quality issues identified in the manufacturing process resulted in a subset of product being out of specification – in other words having an insufficient quantity of active drug product. FDA reviewed applicable scientific data and determined that certain lots of these auto-injectors can be used for an additional period of time beyond their original labeled expiration date, if properly stored. In addition, to address a temporary delay in delivery of replacement potassium iodide product - which can help block radioactive iodine from being absorbed by the thyroid gland, thus protecting this gland from radiation injury – to eight states, FDA authorized the use of specific lots of properly stored potassium iodide for up to an additional six months beyond the manufacturer's labeled expiry date.

In addition, in 2013, even before PAHPRA was enacted, FDA addressed state and local stakeholders' concerns about Tamiflu expiry by concluding that based on FDA's review of scientific data, provided the products have been stored under labeled storage conditions, it is scientifically supportable for certain lots of Tamiflu capsules held in strategic stockpiles to be used for a maximum of ten years beyond their date of manufacture. FDA had previously addressed similar stakeholder concerns by allowing use of properly stored Tamiflu capsules for up to seven years beyond their date of manufacture following the 2009-2010 H1N1 influenza pandemic.

Moving forward, FDA will continue to use the PAHPRA expiration dating authority to respond to critical medical countermeasure expiry dating challenges at the state and local levels where feasible. FDA has established an internal working group to assess broader policy and procedural considerations with respect to implementing the new expiry dating authority to maximize potential applications to state and local stockpiles, including what additional guidance might be needed to clarify applicable expectations related to expiry dating extensions. Issues this working group are addressing include the scope and source of scientific data needed to support extensions; appropriate requirements and conditions for storage, sampling, recordkeeping, periodic testing or retesting, product disposition, and labeling of extended products; and, approaches to potential local and state requests for expiry dating extensions from FDA.

MOST LIKELY THREATS

Ms. DELAURO. Final question is what are the most likely threats in your estimation? What are they? And it is important to get an understanding of not scaring people. That is not the point, but how prepared are we to deal with this?

There has got to be a certain level of, I want to say honesty, just focus on if we are or if we are not. And if we are not, why not? But in your view, and I am happy to have people, you know, everybody, you know, talk about what they view what are the threats? What are the most likely threats to emerge not just for 2014, but over the next several years—natural, manmade, or whatever—and how prepared are we?

Go for it.

Mr. KINGSTON. Yes, and let me—let me elaborate on that a little bit. But kind of putting it in a context of the question I asked you about the pandemics in the past, but what if members of this panel wanted to give a speech, you know, a 5-minute on here is what the danger is, here is where we stand. I think that would be useful for all of us so that we are just able to translate this to the next rotary club.

Mr. KORCH. Well, to answer your question, I would love to know that as well. If I could predict what the next major emerging disease was going to be, I think I would be making a lot of money just in that prognostication. But that is not to make light of it.

I think what we can see is, historically, we know that influenza, and we are seeing that on a regular basis, all these—

Ms. DELAURO. You all are very professional people. You have opinions as to whether or not—what direction we ought to move in here.

Mr. KORCH. So I know that flu is constantly on the horizon. It is, as you can see, historically as well as in current times, with agricultural practices, with all the things that we are doing, flu still remains a present and omnipresent threat.

The manmade or the deliberate threats, outside of having perfect knowledge of what an aggressor tends to want to do, we know that the threats that we are working on are the ones that have emerged historically as the ones that present the biggest challenges or have the biggest impact potentially.

The other threats that emerge, the coronaviruses that come on up, the morbilliviruses, all these others, we are looking not so much at do we have something specific that we can address with that particular threat, but as you have heard all across the board, how we are developing enough resiliency in the products that we are looking at, in the infrastructure that we are putting in place, so that we could turn that crank very quickly?

It is not a function of predicting exactly what the threat is going to be. It is more of a function of having this infrastructure to rapidly respond when the opportunities start arising, when we see, as early as we possibly can—either through surveillance or through information—this capability established across all these partners in terms of rapid manufacturing, rapid identification, the recombinant technologies, the platforms that you have heard about.

That is really it, going from what is the prediction to what is the real innate capability to respond.

Mr. KINGSTON. Let me ask you on that. We know that Assad has used chemical weapons. Do we—if that had been used on Americans, what would have happened?

Mr. KORCH. Hard for me to project what would have happened under those circumstances. We certainly know that his use of nerve agents, we have prepared to the extent that we have with the CHEMPACKS that are forward placed in a number of localities in the United States. That there are countermeasures for nerve agent, for cyanide.

To our knowledge of what is a potential threat, we have provided materials, especially on the chemical side, as far forward as possible because you don't have the luxury of a lot of time to develop a response. They have to be there when the event happens.

And that has been the strategy that we have used, to forward position those particular medical countermeasures at hospital settings and in localities identified by the State and locals and through CDC's Strategic National Stockpile to be as far forward as possible.

And to the extent that we can understand what the next range of chemical threat would be, we would be looking at what we can do to mitigate that as well. But the scenario you build with Assad's or the occurrences in Syria certainly are ones that keep us awake with regard to are we prepared?

You also heard from Robin that there is new products that have come down the line with regard to anticonvulsants, and midazolam replacing what is currently in the stockpile.

So we are always looking to upgrade and asking how can we make it easier to prepare, to have responses, to administer? Those are all components. Safety and efficacy of the products always are on our mind in terms of what we can do to respond.

Mr. KINGSTON. Go ahead.

Ms. DELAURO. No, I just wanted—is there concurrence on the issue of influenza as—

Mr. ROBINSON. I would not only include influenza, but there are other emerging infectious diseases that we know is not a matter of if, it is just a matter of when.

And Dr. Korch said we can't make a medical countermeasure for all 255 or 296 different pathogens, but what we can do is we can have the infrastructure that did not exist 10, 15 years ago to be able to do what we did for H7N9, and that is to rapidly be able to take products, candidates that are very early in the pipeline and move them rapidly forward and which we can actually have those available.

Something BARDA actually moved forward with in MERS coronavirus, with a drug that is licensed actually for parasitic infections. We had been studying it for influenza, and we said, you know, this might work for MERS coronavirus and then worked with CDC and the NIH to actually determine whether or not it may be an effective drug for MERS coronavirus.

Ms. DELAURO. Anyone else want to just end the conversation, or is that final? FDA?

Dr. KURILLA. The point I would make is that just as you mentioned with universal flu, if you are constantly looking or trying to predict where the next flu pandemic is going to arise from, the solution is rather than to get more refined and be very clever about predicting is to just come up with a solution like the universal flu that doesn't matter if you have something that you know will work. And we are on the cusp of a lot of scientific opportunities that offer that broad spectrum potential, which we have seen in terms of antibiotics and bacterial diseases.

We are on the cusp of being able to address that from antiviral capabilities where one drug might actually be effective against a wide variety of viral threats. So as a new one emerges, we may end up already having something that people are using for other things that will be effective.

Ms. DELAURO. Sure. Sure. It is like great research with—you know, cancer research. You can use it both—anyway. Anyone else? Because go ahead. FDA?

Dr. BORIO. I think that assessing the threat is one of the most extremely difficult tasks we have, which is why again just to reiterate, you know, we do need very nimble response mechanisms that focus on resilience. Also we talk a lot about handoff from one place to another, but the truth is that we work together all the time, and engagement continues. So FDA, for example, is engaged from start to finish.

Just next month, we are having NIH researchers come to FDA to present animal models of respiratory diseases. Traditionally, we would have waited for a product. You know, this is now how we work today. We are there very early on, and if I dare to say, we have matured together as professionals, and we actually like each other very much. [Laughter.]

NATURAL VS. MANMADE THREATS

Ms. DELAURO. That is good. That is always good.

I just want to say—and Mr. Chairman, Dr. Harris—it would seem from the commentary, and it is not there because manmade is out there. But the fact is, is that where some of the dialogue and the conversation has been is around naturally caused diseases here.

And I think it is important in terms of how we view the resources that we utilize because in the past, and maybe even now, the pot of money has been skewed to manmade threats, at least with regard to, I believe, BioShield, that that money then is for that side of it, the kind of manmade threat assessment versus the infectious disease side of the equation.

I think as part of the overall—because when I asked my first question about where you create the balance between the two, we have to take a look at where this data and your professional capabilities are carrying you so that we are placing resources in the right buckets here, or maybe there needs to be more flexibility as to where this goes, depending on how this is moving. This is not static. This is fluid here.

Mr. ROBINSON. As you have pointed out, you are spot on on this. We have to make the medical countermeasures that we are devel-

oping for under BioShield have more applicability to public health and the community of diseases.

Case in point. The dollars that we are putting into developing new classes of antibiotics that are going to work against biothreats like glanders, melioidosis—

Ms. DELAURO. Glanders, yes.

Mr. ROBINSON [continuing]. Plague, tularemia, they are also being looked at for MRSA, CRE, and other hospital- and community-acquired infections that are rampant out there. And that is an approach across the board that we are doing, and so what you are seeing is these drugs will be doing double duty not just for Project BioShield and biothreats, but also for public health emergencies and just everyday pathogens.

Ms. DELAURO. Mr. Chairman, thank you very, very much, and thank all of you for your testimony.

Mr. KINGSTON. Thank you.

Dr. Harris, are you finished?

All right. Well, thank you very much for your time and the good work you are doing.

And the Members will have 2 weeks in which to submit further questions or comments, should they have them.

[The information follows:]

Mr. KINGSTON. And we stand adjourned.

Department of Labor, Health and Human Services and Education and Related Agencies

Budget Hearing: Public Health Emergency Medical Countermeasures Enterprise (PHEMCE)

Thursday, February 27, 2014

Questions for the Record from Chairman Kingston

The Process to Coordinate

The budgets of the organizations in front of us represent about \$2.8 billion of taxpayer resources dedicated to addressing medical countermeasure needs to protect against high priority threats, as determined by the Department of Homeland Security. As we have heard, these organizations are moving toward the implementation of a strategic planning approach to biodefense activities.

1. To All -- I would like each witness to identify what policies or procedures at your agencies had to change to allow for this more coordinated approach and use of a multiyear budget process that is linked to a multi-agency strategic plan?

Response (ASPR): The Office of the Assistant Secretary for Preparedness and Response (ASPR) has spearheaded the effort to develop a multi-agency budget forecast, in response to internal directives that emerged from the 2010 Medical Countermeasures Review, and more recently to address the requirement stipulated in the 2013 *Pandemic and All-Hazards Preparedness Reauthorization Act* (PAHPRA). The ability to develop multiyear budget projections did not require radical changes in policies and procedures. Rather, it required a different perspective or understanding of the financial impacts that each organization imparts on other partners within the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE).

A strong element of multiyear planning runs through the PHEMCE Strategy and Implementation Plan, since it uses a multiyear horizon to direct implementation of a range of activities. We have annually conducted the Strategic National Stockpile (SNS) Review, which forecasts budgetary planning three years in advance of the current fiscal year, and which requires a coordinated approach among the PHEMCE partners to project needs and resources. Each agency also has individual Strategic Plans, many of which have already been informed by the overarching objectives of the PHEMCE. Therefore, we linked budgetary commitments and projections from these individual plans and from the overarching PHEMCE strategy to develop a multiyear budgets. The most meaningful outcome of this new process, was the assessment of products or projects projected to transition from agency to agency. This information will enable ASPR and other PHEMCE to plan more strategically for biodefense activities.

ASPR's Biomedical Advanced Research and Development Authority (BARDA)—a member of the PHEMCE—participates fully in the ASPR-led multiyear budget development and PHEMCE strategic planning processes. From its inception in 2007, BARDA has been aligned with the PHEMCE's coordinated approach and has included PHEMCE partners in planning,

implementation, and oversight of its chemical, biological, radiological, and nuclear (CBRN) and pandemic influenza medical countermeasure (MCM) programs and projects. BARDA has used multiyear budgeting as a best business practice since 2008 and has shared its budget forecasts with PHEMCE partners since 2011. Therefore, BARDA is experiencing minimal change because of the PHEMCE coordination and multiyear budgeting.

Response (NIH): The National Institutes of Health (NIH) uses many financial systems, databases, and processes for the planning, forecasting, management, review, and approval of projects comprising its Biodefense and Emerging Infectious Diseases research portfolio. In order to improve coordination with the Public Health and Emergency Medical Countermeasures Enterprise (PHEMCE) multi-year budget, NIH consolidated and categorized its research activities to effectively align with the PHEMCE portfolio's strategic goals, objectives, threat areas, and portfolio classifications. NIH also created financial models that forecasted how resources would be deployed among PHEMCE's threat areas assuming three different funding scenarios outlined in the multi-year PHEMCE budget. These models incorporated existing financial commitments, new and expanding areas of research, HHS and NIH programmatic priorities, and anticipated inflation.

NIH has worked and will continue to work closely with the Office of the Assistant Secretary for Preparedness and Response (ASPR) to ensure the multi-year budget is consistent with PHEMCE priorities. NIH and ASPR maintain regular communication regarding programmatic priorities, research advances, and anticipated product transition dates to ensure smooth scientific progression between agencies. In response to the multi-year budget request, NIH enhanced its review of planned product and/or project transitions to BARDA or other downstream partners in the product development pipeline.

Response (FDA): FDA has not had to make any changes to its policies or procedures to coordinate its Medical Countermeasures Initiative (MCMi) with the PHEMCE multi-year budget process. Since the MCMi was launched in 2010, FDA has worked to ensure that the program is effectively coordinated with PHEMCE priorities and objectives and that MCMi programs are sufficiently resourced and aligned to facilitate the fulfillment of program goals and objectives. The PHEMCE multi-year budget process enables FDA to use its professional judgment to estimate out-year MCMi resource needs in coordination with its PHEMCE partners, which is used to inform annual budget formulation priority setting.

Response (CDC): CDC has developed and maintained multi-year projections of requirements to sustain the Strategic National Stockpile (SNS) and replace expiring SNS assets based on life cycle cost modeling since 2010. The incorporation of these projections into the PHEMCE multiyear budget activity as part of our participation in the interagency process required no changes for CDC policy or procedures.

2. Dr. Robinson -- In part the Public Health Emergency Medical Countermeasure Enterprise (PHEMCE) works to facilitate and encourage the private market to develop vaccines, therapies, and diagnostic tools for public health medical emergencies. How does the PHEMCE process ensure programs do not overlap with what industry is already willing or considering to develop without federal funding?

Response (ASPR): ASPR/BARDA utilizes multiple outreach approaches including strategic plans, BARDA Industry Day, solicitations, Tech Watch, and our websites to communicate our medical countermeasure priorities and activities to industry stakeholders and hear from industry about their products and business issues. The HHS, PHEMCE, ASPR, and BARDA Strategic Plans each articulate the medical countermeasures mission space and requirements for external stakeholders at a high level. The PHEMCE further defines these programs through the individual requests for proposals (RFP), acquisition processes, and use of public forums to communicate specific requirements (e.g., BARDA Industry Day).

Separate from the formal acquisition process, the ASPR/BARDA TechWatch program provides a direct opportunity for industry to share information about current projects under development and receive feedback on their alignment with PHEMCE requirements. To date, BARDA has held more than 500 separate meetings, and this forum serves as an opportunity to share information and provide insight into industry activities. It also helps to ensure that there is visibility on funded programs in both the public and private sectors.

As a regular business practice, ASPR/BARDA conducts market analysis on industry sectors (e.g., vaccines) to develop product landscape maps and company and product profiles, including company capitalization. This comprehensive due-diligence approach affords BARDA opportunities to engage companies on their product development pipeline and portfolios, and the associated risks and challenges toward reaching licensure and marketing alone or with U.S. government and/or other third party investment (e.g., venture capital). Additionally, BARDA reaches out to the end-user health care community as a standard business practice to discuss the utility and acceptability of existing and future medical countermeasures under development. For example, talks with the American Burn Association and burn center physicians have provided significant benefits to BARDA's thermal burn medical countermeasures program.

Administration Commitment to Process

1. **Dr. Korch:** Now that PHEMCE has coordinated a strategic plan and multiyear budget, please explain if or how OMB and the Secretary have committed to ensure the bio-defense readiness and stockpile capacity are proposed in the budget request at the same level identified in the five year plan?

Response (ASPR): The Secretary of Health and Human Services called for the need for both strategic planning and for a multiyear budget as part of a larger body of improvements identified in the 2010 Medical Countermeasures Review. HHS and the Administration are committed to preparing for public health emergencies, as evidenced by the development of these tools to better inform resource planning. These documents delineate what goals and objectives we seek to achieve and provide a target for programming funding levels that would be needed to complete projected investments in medical product development or acquisition up to a level that we believe is the theoretical need for full preparedness.

The Department and the Administration recognize that competing demands for resources will require prioritization. The strategic plans and multiyear budgets represent HHS' best plans and estimates at any given time for PHEMCE program execution and they help to inform the

President's Budget. The analysis is provided without future regard to the competing priorities that the Secretary, other HHS officials, and the President, must consider as the President's budget is developed. and that Congress must consider in preparing and passing appropriations legislation. These analyses of future budget needs to achieve these preparedness goals do serve to assist the Department, the Administration, the President, and Congress in fulfilling their obligations.

Process for Portfolio Analysis

Given the \$2.8 billion spent by PHEMCE agencies, there is obviously the potential for overlap of activities.

1. Dr. Korch: Please provide examples of the cross-agency portfolio analysis used to prevent duplication of effort?

Response (ASPR): PHEMCE efforts and investments span a wide-range of medical products, different threats, and different levels of activities in the maturation of any given product. The close coordination of the many projects or contracts within the PHEMCE—through the mechanism of biweekly meetings of the Enterprise Executive Committee, the periodic portfolio reviews of work being conducted on MCM efforts, the SNS annual review and the upcoming Portfolio Tracking Tool—all combine to reduce the chance of duplication of effort.

Specific areas that highlight how limited resources are maximized through this coordination process can be found in MCM development for radiological and nuclear threats, where the National Institute of Allergy and Infectious Diseases (NIAID) works very closely with ASPR/BARDA to identify the most promising candidate products that would mitigate effects from radiation on the patient, such as lowering of white blood cells leading to increased vulnerability to infection. In this case, we were able to determine that work performed earlier by the National Institutes of Health (NIH) on several products already approved for cancer treatment patients may be applicable to our needs in treating nuclear injuries.

Another area where we coordinate efforts to avoid duplication comes from studies to determine whether our current vaccines that are licensed for anthrax may be able to be used at lower doses or on a different schedule during a time of limited supply. Rather than multiple different agencies addressing the issue, we combined energy and resources to come up with a strategy that engaged the Centers for Disease Control and Prevention (CDC), the Food and Drug Administration (FDA), BARDA and NIAID to agree on scientific studies and then to jointly evaluate the data that may permit us alternate strategies for vaccine use under contingency conditions, or to potentially stretch our supply without additional investments.

2. I would like CDC, NIH, and FDA to discuss how their agencies harmonize the scientific activity across the various agencies to ensure scientific gaps are filled and to minimize overlap?

Response (NIH): The National Institutes of Health (NIH) maintains a strong and active presence within the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) by participating in multiple teams and committees to ensure coordination of scientific activity with PHEMCE partners. These include Integrated Program Teams that coordinate efforts on particular biodefense and chemical, radiological, and nuclear threats, or in cross-cutting portfolio areas such as diagnostics; the Enterprise Executive Committee that addresses policy and

program-level issues; the Portfolio Advisory Committee of the Integrated Portfolio for CBRN Medical Countermeasures that aligns Department of Health and Human Services (HHS) and Department of Defense medical countermeasure development and infrastructure resources; and the Enterprise Senior Council that provides coordinated, strategic direction and policy oversight. PHEMCE-vetted and approved civilian medical countermeasure requirements are a key factor in identifying the highest priority medical countermeasure gaps and aligning HHS-supported research, including that occurring at NIH, to address them. NIH research priorities in support of this medical countermeasure development were detailed in the 2012 PHEMCE Strategy and Implementation Plan (SIP), and will be updated as needed in future iterations of the SIP. In addition, ASPR leads the PHEMCE in conducting comprehensive threat-specific portfolio reviews every 18 months to evaluate major programs and future action items, provide strategic coordination, and align activities to address scientific needs and avoid duplication. These efforts ensure dialogue between agencies to promote alignment of scientific goals and continuity of purpose.

The NIH coordinates with industry and PHEMCE partners to ensure that the results of NIH-supported research can be translated rapidly into safe and effective medical countermeasures. In particular, NIH collaborates with interagency partners in order to address scientific needs and respond to emerging public health situations. For example, NIH worked closely with the Food and Drug Administration (FDA) to develop a non-human primate animal model to support licensure of antibiotics for plague. During the 2009 H1N1 influenza pandemic, HHS launched a coordinated response, with efforts from FDA, the Centers for Disease Control and Prevention, and NIH, in order to provide safe and effective countermeasures to the public.

Response (CDC): As a component of PHEMCE, CDC is working closely with PHEMCE partners, to manage the development, review, and publication of guidelines for safe and effective utilization of stockpiled medical countermeasures (MCM). As such, CDC's scientific activities are focused on understanding the use and efficacy of MCMs that are not part of routine clinical practice. CDC is addressing the priority items on this list of MCMs through focused teams of subject matter experts, such as the Anthrax Management Team, and the newly formed Radiological Management Team. These teams manage agency activities related to their focus area and identify potential for duplicative activity outside of CDC through participation of team members in the PHEMCE governance process.

CDC also participates in interagency portfolio reviews, to discuss what scientific work is being done at each agency and how we can better leverage partnerships with one another including the Portfolio Advisory Committee meetings that discuss the scientific agenda of each agency.

Response (FDA): FDA collaborates extensively with PHEMCE and Department of Defense (DoD) partners to foster the development and availability of medical countermeasures (MCMs) including providing subject matter expertise and technical assistance to numerous standing Enterprise- and DoD-specific committees and working groups that develop MCM requirements, plans, priorities, and policies and conduct program oversight and integration. In addition, to ensure that the MCM Regulatory Science Program – established under FDA's Medical Countermeasures Initiative (MCMi) – is appropriately targeted and coordinated with U.S. government MCM priorities, FDA established a Steering Committee for Advancing MCMi

Regulatory Science – which includes representatives from NIH, CDC, BARDA, and DoD – that evaluates MCMi Regulatory Science Program research proposals for scientific/technical merit and feasibility as well as for alignment with Enterprise priorities.

3. Dr. Robinson: How often does PHEMCE review its portfolio analysis process?

- a. When was the last review and what lessons learned came out of the effort to improve coordination of effort across the enterprise.

Response (ASPR): The PHEMCE has conducted portfolio reviews on an eighteen-month cycle since 2010 for the smallpox, anthrax, radiological and nuclear threats, botulism, broad spectrum antimicrobials, chemical and pandemic influenza MCM portfolios. Separately, since 2008, ASPR/BARDA has conducted annual product portfolio reviews using a formal portfolio review process based on pharmaceutical industry standards, metrics, and methodologies; and an external scientific panel for guidance on existing programs and new initiatives.

So far in 2014, the PHEMCE has conducted three portfolio reviews on MCM areas: anthrax, radiological and nuclear threats, and pandemic influenza. A common theme among all three of these portfolio reviews is the abundance of solid progress and effort on priority action items that emanated from the previous portfolio reviews, especially with regard to the development, acquisition, licensure, and stockpiling of MCMs for these threats. The major areas garnering attention now are how we distribute, administer, and monitor the safety and performance of these MCMs going forward with the existence of these products.

Relationship with HHS and DoD Approach to buy Medical Countermeasures

My understanding is that DoD also purchases medical countermeasures and participates, at some level, within PHCEME.

1. Dr. Korch and Dr. Robinson -- Please explain how DoD works with PHCEME in the development of medical countermeasures.

Response (ASPR): The Department of Defense (DoD) is primarily engaged with the PHEMCE through the Office of the Assistant Secretary of Defense for Chemical and Biological Defense (ASD(CBD)). DoD participates as a voting member at every level of the PHEMCE, from the subject matter expert level on the Integrated Program Teams to the most senior level on the Enterprise Senior Council. ASPR/BARDA has met monthly with CBD leadership since 2011 to discuss strategic planning and programmatic events, activities, issues, challenges, and solutions in the development, manufacturing, acquisition, and licensure of MCMs. Additionally, HHS has entered into agreements to procure and store certain medical products on behalf of DoD, such as smallpox and anthrax vaccines.

With regard to product development, there are three major areas of cooperation and communication. First, HHS and DoD have set up a committee of federal officials called the Portfolio Advisory Committee (PAC) for the Integrated CBRN Medical Countermeasures Portfolio under the auspices of the PHEMCE. The PAC is co-chaired by senior representatives from HHS/ASPR and DoD/CBD. The group explores the areas of common interest and need across the two portfolios and, wherever possible, seeks to leverage each other's capabilities to

achieve economy in developing specific MCMs, such as for hemorrhagic fever virus treatments, defense against chemical threats, and products to treat radiation injury. Another major area of cooperation has been in establishing a computer-based management system called the Portfolio Tracking Tool, whereby each organization provides data on specific projects and programs under contract to track progress across multiple products to identify how specific requirements are being achieved in product development, from late research through product approvals by FDA.

Secondly, each organization has visibility regarding the other's program in establishing the respective Centers for Advanced Development. These are high-value, public-private ventures established with pharmaceutical leaders to assist in advanced development and surge capacity for biodefense and pandemic influenza products. The DoD and HHS Centers each have unique capabilities that can potentially be leveraged to accommodate the needs of the other program. Coordination has been established to maximize this potential.

Finally, DoD and HHS are members of an international organization termed the Chemical and Biological Defense Memorandum of Understanding. This body coordinates biomedical product development among the defense and public health organizations of the United States, Canada, Australia, and the United Kingdom. A subgroup, termed the Medical Countermeasures Committee, works on developing products such as point-of-care diagnostics, novel antibiotics, and MCMs against chemical and toxin threats that are of mutual benefit among the signatory nations.

A consolidated consideration of this coordination between HHS and DoD is available in the GAO Spring 2014 report, GAO-14-329, which is available at <http://gao.gov/assets/670/662120.pdf>.

2. Dr. Korch and Dr. Robinson – How do DoD and HHS approach procurement of medical countermeasures? Specifically, do they use the same process and contracts? If they use different procedures, why is that necessary?

Response (ASPR): DOD and HHS approach procurement of MCMs in generally the same fashion. Both organizations use contractual mechanisms permitted under the Federal Acquisition Regulation (FAR), although there may be slight differences promulgated under departmental derivatives of the FAR (such as the Defense Federal Acquisition Regulation Supplement). Rarely do we use the same contracts. However, there are examples of business interactions where early work was funded by the DOD and then follow-on work was performed under contract with HHS as part of the transition from early to advanced product development (e.g., gastro-intestinal [GI] tract radiological/nuclear MCMs).

Both departments also have authority to procure goods and services under Other Transaction Authority (OTA). OTAs permit acquisitions that do not follow typical contracting rules laid out in the FAR. In general, OTAs provide greater flexibility in these business relationships with vendors. Within HHS, ASPR/BARDA follows accepted practices of soliciting proposals either through Broad Agency Announcements or specified Requests for Proposal. CDC's Division of Strategic National Stockpile uses well-established federal contracting vehicles and procedures for procurement of licensed, commercially-available products to add to the federal stockpile.

NIH is generally concerned with funding basic research that may lead to future products and solicits grant proposals through a well-established mechanism involving a highly-refined, peer-reviewed system for grant approval. National Institute of Allergy and Infectious Diseases (NIAID) can also issue contracts for specific goods and services using standard procedures described above.

Despite the similarities, HHS and DoD differ somewhat because we are driven by different requirements. HHS' MCM acquisition contracts for CBRN threats are driven by requirements and stockpiling goals that are informed by material threat assessments from the Department of Homeland Security and deliberated and approved by the PHEMCE. DOD, on the other hand, derives its requirements for and approves acquisition for MCMs based on internal DOD processes. On occasion, HHS and DOD do use the other department's contracts for purchasing MCMs (e.g., antiviral drugs), where it is the best value for the government to do so. For example, both DOD and HHS have invested in the development of drugs to treat individuals symptomatic with smallpox, sharing the costs for development of these products. HHS and DOD share a common stockpile of anthrax vaccine and smallpox vaccine.

Regulatory Science

We understand that the process to speed the development of medical counter measures requires close coordination with FDA. These efforts may also require an increased focus on regulatory science activities. I would like to understand how the PHEMCE organizations work to leverage their efforts with other programs that support similar federal goals. Specifically, NIH has a program called the Cures Acceleration Network (CAN). It is expected to support activities that accelerate therapeutics to market. I am not aware of any reason that CAN could not test or support regulatory science projects designed to increase the speed that FDA approves new therapeutics related to countermeasures, which would allow for HHS to meet two objectives at one time.

1. Dr. Korch: Has PHEMCE proposed any projects to NIH's Cures Acceleration Network (CAN) that could be used to test any processes or procedures that would speed up FDA's process to get drugs to market and support PHEMCE needs at the same time?

Response (ASPR): The PHEMCE has not proposed any projects to NIH's Cures Acceleration Network (CAN). The CAN was established to provide opportunities for grant awards to advance the development of highly needed cures, ostensibly for naturally-occurring disease conditions of a rare or difficult nature, where there has been a barrier between discovery of novel candidate products and clinical trials. Project awards may use other transaction authorities and may require partnership matching funds. A principal objective of the CAN is to identify mechanisms or products that might speed transition of a medical product through the regulatory process. These opportunities may be as a result of conducting regulatory science to develop standards for development, manufacturing, and clinical utilization of diagnostics, reagents, and devices. Acceleration instead may result from other mechanisms established with the regulatory agency to address a high profile need.

The PHEMCE has parallel issues with regard to accelerating the pace of development and regulatory review for these critically needed medical products to treat fundamentally different

needs, but has successfully approached this in a different way. PHEMCE has been able to work with FDA to address these PHEMCE-unique needs and achieve similar outcomes by virtue of agreements and programs that arose from the 2010 Medical Countermeasures Review. As for the advanced development process, ASPR instituted a process called the In Process Review (IPR) for each MCM contract that BARDA issues. The IPR is overseen by the senior program official (typically the BARDA Director) and it is a formal assessment of the progress, costs and deviations for each contract, leading to a decision on continuation, improvement or cancellation based on a detailed presentation of the status of the projects by the industry and government officials. At the basic research level, the National Institute of Allergy and Infectious Diseases (NIAID) established a program called the Concept Acceleration Program (CAP), which serves to identify very promising candidate products or concepts that deserve additional funding to help mature the products to a point where they may be ready for BARDA or other advanced development opportunities. On the regulatory front, the FDA developed the Medical Countermeasures Initiative (MCMi) to directly address concerns about the clarity and speed of the regulatory process for the medical products developed for CBRN threats. The MCMi instituted three major actions to improve the process for PHEMCE-related products. First, MCMi identified and assigned key senior reviewers to help accelerate regulatory oversight of these products. Second, MCMi invested funds to improve regulatory science capacity at FDA and improve interaction with the product sponsors on specific technological issues. Third, MCMi requested legislative changes that would speed availability of candidate products to the public during times of urgent need. Furthermore, in the 2012 PHEMCE Strategy and Implementation Plan, there is a specified goal to improve the regulatory process, and the PHEMCE tracks specific implementation of a range of activities to achieve this end. This initiative helps achieve similar outcomes to those identified by the CAN. Overall, these combined approaches have resulted in a much improved level of successful translation of candidates into approved products. However, the PHEMCE is still interested in other means to improve or to take advantage of different means to accomplish its goals, so it will further explore opportunities with the Cures Acceleration Network.

2. Dr. Korch: If not, can you look at how PHEMCE might work with CAN to leverage both programs common objectives?

Response (ASPR): Although the PHEMCE has not directly engaged the CAN to date, we will pursue discussions on whether there is an opportunity to leverage each other's processes and capabilities to realize improvements in common objectives and speed the availability of life-saving medical products to the public.

FDA Approval Process

1. Mr. Burel: Of all new countermeasures procured by BARDA in the Strategic National Stockpile, what percentages are approved by the Food and Drug Administration?

Response (CDC): 75% (9 of 12) countermeasures added to the SNS through BARDA procurement have been approved by the FDA; however, only 5 of 12 (42%) are FDA approved for their intended use. Therefore, 7 of 12 (58%) require an Emergency Use Authorization in order to be used during an incident.

2. Dr. Borio or Dr. Robinson: How long does it typically take for a new countermeasure to be approved by FDA after BARDA initially procures it?

Response (FDA): How long it takes for a medical countermeasure (MCM) procured by BARDA under Project BioShield using the Special Reserve Fund (SRF) to be approved or licensed by FDA depends on the particular countermeasure, the regulatory pathway to approval or licensure, and the stage of development at the time it was procured. For MCMs not yet FDA approved or licensed that BARDA wants to procure using the SRF, statute requires that there be “...*sufficient and satisfactory clinical experience or research data (including data, if available, from pre-clinical and clinical trials)* [to] *support a reasonable conclusion that the countermeasure will qualify for approval or licensing within 10 years...*” or that the MCM be authorized for use under an Emergency Use Authorization [42 U.S.C. § 247d-6b]. There are two MCMs that were investigational when they were procured by BARDA using the SRF that have since achieved approval or licensure: (1) Botulism Antitoxin Heptavalent (A, B, C, D, E, F, G) (Equine) (BAT) which is licensed for the treatment of symptomatic botulism following documented or suspected exposure to botulinum neurotoxin serotypes A, B, C, D, E, F, or G in adults and pediatric patients; and (2) Raxibacumab, which is approved to treat adult and pediatric patients with inhalational anthrax in combination with appropriate antibacterial drugs and to prevent inhalational anthrax when alternative therapies are not available or not appropriate. The time from the initial SRF procurement contract to approval or licensure for these MCMs was 6.5 years and 7 years respectively. However, these times from initial procurement contract to approval or licensure should not be considered typical. The time it takes from the issuance of an SRF contract to approval or licensure depends on many factors related to developing the scientific data necessary to enable regulatory decision making, which can often present complex and difficult challenges. FDA works closely with BARDA and product sponsors/applicants to facilitate MCM development and regulatory review as quickly as practicable.

Response (ASPR): The time between ASPR/BARDA’s support of MCM candidates for advanced development (i.e., Phase 1 clinical trial completion) and FDA approval typically ranges between six and nine years, depending on many factors including product safety, efficacy, and the manufacturing process. Under section 319F-2 of the Public Health Service (PHS) Act as originally added by the Project BioShield Act of 2004, MCM products could be acquired under Project BioShield only if there was a basis for a reasonable conclusion that the countermeasure would qualify for approval or licensure within eight years. Under that section as amended by PAHPRA, this timeframe was extended to ten years. The two CBRN MCMs that have received FDA approval recently and after having been supported by Project BioShield are an anthrax antitoxin, Raxibacumab®, and the heptavalent botulinum antitoxin, HBAT®. BARDA’s support of late-stage development of Raxibacumab® began in September 2005. FDA approved the product in December 2012—just over seven years later. BARDA started development support of HBAT® in September 2006, and FDA licensed it in March 2013—about 6.5 years later. Other MCMs (e.g., anthrax vaccine for post-exposure prophylaxis) purchased under Project BioShield may take longer than these two antitoxins for FDA licensure. All of the MCMs procured under Project BioShield that are not licensed or approved are available from the SNS or the manufacturer’s inventory under an Emergency Use Authorization (EUA) in an emergency, as HHS does not stockpile the MCM until there is sufficient data supportive of its usage under an

EUA as determined by the FDA.

3. **Dr. Borio:** I understand that there is a unique challenge facing FDA in terms of the ethics of challenging humans with deadly viruses like smallpox or anthrax, so using animal models is the only plausible means to test these countermeasures. With that said, can you explain what goes into FDA's approval process for countermeasures that BARDA has procured and why it takes as long it does to secure FDA approval for these countermeasures?

Response (FDA): To be approved for use, an MCM must be determined to be safe and effective for its intended use. All of FDA's regulatory mechanisms to enable access to MCMs are based on benefit-risk assessments that are founded on available scientific evidence of safety and effectiveness, as well as many other factors that affect benefit-risk assessments. Safety is always based on data from clinical trials in humans, as well as data from non-clinical studies. Efficacy is also based on clinical trials in humans when feasible, but when it is unethical or infeasible to conduct human efficacy studies, FDA relies on adequate and well-controlled animal studies. FDA also evaluates information regarding consistency of manufacturing, purity, potency, stability and sterility to ensure that MCMs meet manufacturing and quality standards. Other factors that affect benefit-risk assessments that are considered include the nature and severity of the condition the MCM is intended to prevent or treat, the benefits and risks of other available therapies for the condition, and any risk management tools that might be necessary to ensure that the benefits of the MCM outweigh its risks.

The length of time it takes to achieve approval or licensure of MCMs is directly related to gaps in scientific knowledge associated with MCMs that create unique and complex challenges with respect to developing the data necessary to support regulatory decision-making. These gaps in knowledge arise from multiple sources including intrinsic uncertainties about the biologic behavior of threat agents in potential public health emergencies. For example, there is often an incomplete understanding of the nature and severity of the conditions for which MCMs are being developed to prevent or treat because many of these threats do not occur naturally or they occur at such a low incidence that it is nearly impossible to study them in humans. Additionally, because many of the high-priority threats for which MCMs are being developed do not occur naturally to an extent that would support the conduct of field efficacy studies in humans and it is not ethical to conduct human challenge studies with such threat agents, FDA must often base its regulatory decisions on the extrapolation of data from animal studies to humans. However, for many threats there are not adequate animal models to support MCM development or sufficient biomarkers to enable the extrapolation of data generated in animal models to man. Without such tools it is difficult to create the data necessary to support regulatory decision-making.

FDA works closely with its PHEMCE partners and product sponsors/applicants to facilitate MCM development and regulatory review. In addition, FDA supports a robust Regulatory Science Program under its MCMi to develop the tools, standards, and approaches to assess MCM safety, efficacy, quality, and performance and to help translate cutting-edge science and technology into innovative, safe, and effective MCMs.

NIH/BARDA Collaboration

We are all familiar with the work NIH does, particularly as it relates to basic biomedical research.

Dr. Kurilla: Can you tell me how the basic research portfolio at NIAID assists BARDA in terms of helping to spur the development of countermeasures? Are there any examples you can cite of success stories where NIAID funded research has led to countermeasures that BARDA has procured?

Response (NIH): The National Institutes of Health (NIH) supports foundational research and facilitates interagency partnerships that lead to the development of new and improved medical countermeasures against biological, chemical, radiological, and nuclear threats. The National Institute of Allergy and Infectious Diseases (NIAID) is the lead component of the NIH for research and development of these medical countermeasures. Basic research supported by NIAID contributes to a comprehensive understanding of the scientific and medical aspects of potential threat agents, including newly emerging and re-emerging infectious agents as well as chemical, radiological, or nuclear agents.

The NIAID research portfolio includes basic research to understand the biology, immune response, and pathogenesis of potential bioterror agents and emerging infectious diseases, including influenza, tularemia, plague, and smallpox. This research provides insight into how these agents cause disease and reveals potential targets for the development of medical countermeasures to diagnose, treat, and prevent disease. NIAID also supports translational and product development efforts to capitalize on scientific discoveries and novel concepts identified by basic research and to advance candidate medical countermeasures through the product development pipeline.

NIAID aims for our basic research investment to inform development of products that ultimately can gain Food and Drug Administration (FDA) approval, licensure, clearance, or authorization and be considered for inclusion in the Strategic National Stockpile. If candidate countermeasures show promise in proof-of-concept animal studies or early human testing, NIAID transitions these candidates to the Biomedical Advanced Research and Development Authority (BARDA) for advanced development. Examples of recent successful transitions from NIAID to BARDA include therapies for anthrax and pandemic influenza, a next-generation treatment for chemical exposure, and two smallpox antiviral drugs. NIAID also supported clinical trials, advanced development, and manufacturing services leading up to the BARDA's procurement of Bavarian Nordic's smallpox vaccine, IMVAMUNE®, which has been accepted into the Strategic National Stockpile. In addition, the majority of the candidate medical countermeasure projects at BARDA to mitigate acute radiation syndromes had their genesis at NIAID. Results from recent NIAID studies have supported the procurement of NEUPOGEN® and Leukine® for the Strategic National Stockpile as therapeutics to treat Acute Radiation Syndrome.

Communication with Industry

Dr. Kurilla and Dr. Robinson -- What is the mechanism used to convey PHEMCE related requirements and priorities with industry partners?

Further, can you address how your organizations evaluate the effectiveness of these mechanisms?

Response (ASPR): ASPR/BARDA was mandated by section 319L of the PHS Act, as added by the *Pandemic and All-Hazards Preparedness Act* (2006) to engage stakeholders and communicate requirements and priorities with industry. BARDA's priorities across the CBRN, pandemic influenza, and innovations portfolios are documented in its Broad Agency Announcements (BAAs) and BARDA Strategic Plan (2011), which are published and available through the Federal Business Opportunities (FedBizOpps) and BARDA websites. BARDA's priorities are aligned with the PHEMCE-related medical countermeasure priorities for advanced research, development and procurement through FY17, as published in the 2012 PHEMCE Strategy Implementation Plan, and will be updated as needed in the annual updates to these Plans mandated by PAHPRA. Pre-proposal conference(s) are held to review these areas of interest with industry, and the details of these sessions are available publically. BARDA hosts the www.medicalcountermeasures.gov website, where all HHS MCM solicitations may be found. Additionally, BARDA hosts in-person meetings with industry and academic stakeholders through BARDA's TechWatch program. Currently, from 100 to 150 meetings with stakeholders occur each year.

At least once a year, BARDA hosts "BARDA Industry Day," a multi-day conference bringing together overview presentations of current MCM development priorities. Industry Day also provides the opportunity to meet with BARDA leadership and subject matter experts. NIH is a frequent presenter during BARDA Industry Day on MCM endeavors and collaborations with BARDA, as are other PHEMCE partners. Frequently, NIH and BARDA present their priorities at conferences together. Formal feedback from stakeholders is provided from BARDA Industry Day and TechWatch surveys on BARDA's communication of our priorities, plans, and actions, regular meetings with leadership of stakeholder advocacy organization (e.g., BIO, Alliance for Biosecurity, IDSA, etc.), and frequent direct meetings between BARDA senior leadership and senior leadership of industry partners. Partners are able to provide comments to all levels of BARDA leadership for consideration.

Children, SNS, and Flexible Stockpiling Methods

The Centers for Disease Control and Prevention Director Tom Frieden provided a briefing on this year's influenza activity. It was very helpful when he noted that the first line of defense is vaccination, but, "It's also important to remember that some people who get vaccinated may still get sick, and we need to use our second line of defense against flu: antiviral drugs to treat flu illness." Our focus on vaccination is appropriate, but we don't talk enough on the importance of treatments for influenza, particularly among children. According to the CDC, so far this year 52 children have died from influenza, and 43 percent of children who are hospitalized related to influenza had no complicating medical factor. This means that the flu takes a serious toll on our young and vulnerable which why I am troubled about news reports regarding medications that were in short supply in many parts of the country. We can clearly do a better job of avoiding such shortages by having the government be more flexible on how it approaches the Strategic National Stockpile.

- For example, I understand antivirals purchased for a pandemic are packaged differently than those sold commercially for seasonal flu. This complicates drawing on such a reserve to ease shortages that may occur in severe seasonal flu outbreaks. Has the government thought of working on a system in which the SNS stockpiles antivirals with packaging that could be used

for a normal flu season? I am told that in doing so, we could expedite getting medications to the people who need them while also yielding “fresher” with longer expiration dates. What is the best way to avoid these type of headlines in the future? Are more resources necessary or can this be mitigated by have a better distribution strategy?

Response (CDC): The formulary of the Strategic National Stockpile managed by CDC is currently governed through the PHEMCE process which sets requirements and priorities for SNS procurement. CDC procurement of medical countermeasures addresses PHEMCE priorities for replacement of expiring assets and addition of new products with the available annual SNS appropriation to protect the public health. The missions assigned to the SNS and the associated PHEMCE requirements include resupplying state and local public health and healthcare activities with critical medical countermeasures when existing supplies are exhausted during a public health emergency. CDC has no requirements to procure and hold product in the SNS to intervene in commercial market shortages of commonly available medical countermeasures, and in most cases, does not hold such products or does not hold them in sufficient quantities to significantly impact a widespread commercial shortage.

CDC works collaboratively with other parts of the federal government, manufacturers, other private sector partners, and state and local public health partners to investigate ways in which available SNS assets could be used to alleviate specific instances of commercial shortages. No such plan has been implemented to date.

Deployment of SNS assets to alleviate a temporary commercial shortage could not be accomplished without impacts. If CDC distributed MCM under conditions of a commercial shortage, it is reasonable to expect that replenishment of SNS assets deployed would be delayed by the same conditions that caused the market shortage. Delays in replenishment of the SNS would be compounded by the need to first fill ongoing commercial demand out of existing production capacity. This action could reduce or deplete CDC’s capability to respond to a future public health emergency requiring the same assets. As a result, the United States may be left vulnerable to a specific threat for the months and possibly years required to fully replenish SNS holdings.

Certain products held in the SNS, such as Tamiflu (oseltamivir), were procured specifically for use in a large scale public health emergency. The initial contract for Tamiflu procurement included a clause to ensure that SNS stockpiled antivirals would *not* be released into the commercial market during normal seasonal flu activity, thereby interfering with industry ability to sell antiviral product to customers with the ability to pay. Through discussions with the manufacturer of this product, it has been determined that product purchased under this contract may now be used during a seasonal flu campaign in the event of a market shortage and with the consent of the manufacturer. Follow-on contracts for this product do not include the previous restriction clause.

During the temporary shortage of Tamiflu in December 2012/January 2013, CDC proactively contacted the drug manufacturer (Genentech) to monitor the supply situation.

On January 11, 2013 and January 17, 2013 HHS (through representatives from CDC and BARDA) engaged Genentech's senior Tamiflu team in discussions to determine if additional product was needed to support the US market place. The company confirmed during both meetings that the supply shortage situation was temporary and was projected to resolve in a few weeks. CDC asked the company if they were willing to consider providing supply from the federal Stockpile to backfill US market needs if the shortage did not resolve. The manufacturer stated that if needed, this is something they would be willing to discuss. The shortage resolved within a couple of weeks and the product was not needed.

- Because many younger children (and some elderly) are unable to swallow pills or capsules, treatments that come in liquid form, commonly called "suspension." I am aware the CDC has stockpiled "suspension" formulations of antivirals for pandemic influenza in the past. Can you please tell me how much of this formulation for children is currently in the SNS? How many are we supposed to have?

Response (CDC): The PHEMCE target goals for SNS antiviral suspension (specifically oseltamivir) holdings total 1.6 million treatment regimens; however in an effort to make the best use of taxpayer funds, CDC does not maintain oseltamivir suspension in the SNS. If suspension product was needed during an emergency, there are instructions available for compounding suspension formulation from oseltamivir capsules. To meet the needs of pediatric patients SNS also contains pediatric strengths of oseltamivir capsules (30mg and 45mg capsules). These capsules may also be opened and put into different mediums to facilitate medication administration for children that may not be able to swallow capsules.

- Could we do a better job of easing shortages in the commercial market if the government worked with industry to ensure some consistent level of purchases for the national stockpile, even at a smaller level, thereby keeping production lines open and allowing for production to ramp up quicker as needed?

Response (CDC): The formulary of the Strategic National Stockpile managed by CDC is currently governed through the PHEMCE process which sets requirements and priorities for SNS procurement. CDC procurement of medical countermeasures addresses PHEMCE priorities for replacement of expiring assets and addition of new products with the available annual SNS appropriation to protect the public health. The missions assigned to the SNS and the associated PHEMCE requirements include resupplying state and local public health and healthcare activities with critical medical countermeasures when existing supplies are exhausted during a public health emergency. CDC has no requirements to procure and hold product in the SNS to intervene in commercial market shortages of commonly available medical countermeasures, and in most cases, does not hold such products or does not hold them in sufficient quantities to significantly impact a widespread commercial shortage.

CDC continues to make strategic procurement decisions to smooth and flatten expiration curves of stockpiled MCMs. The end result of this strategy would be a steady, relatively level funding requirement each year to maintain SNS assets and replace expiring products. An additional benefit of this strategy would be the establishment of a steady need for procurement of

stockpiled products. This steady need should provide more consistent, predictable demand for manufacturers maintaining production lines.

Public-Private Distribution Innovation

- 1) I am aware that during the 2009 H1N1 pandemic, the distribution of certain medical countermeasures according to several reports were not as efficient as it could have been. Dr. Korch? What lessons were learned and what steps have been taken to improve the distribution mechanism during times of need?

Response (CDC): The 2009 distribution of pandemic influenza countermeasures, including antiviral drugs from the SNS and state held stockpiles proved that nationwide deployment of medical countermeasures from stockpiled storage was possible and validated that this deployment could be done in the planned timeframes - SNS assets were delivered to all 62 Public Health Emergency Preparedness grant awardees within 7 days.

Although some states and local jurisdictions were able to effectively integrate stockpiled antiviral drugs into the supply chain in accordance with their pandemic influenza plans, other jurisdictions experienced challenges in the distribution or dispensing phases. These challenges identified opportunities which are being addressed through collaborative efforts between CDC, other federal agencies, national stakeholder organizations, healthcare sector representatives, and state and local public health officials. (The HHS Pandemic Influenza Improvement Plan and the GAO Lessons Learned Report provide detailed information on the pandemic influenza lessons learned and areas targeted for improvement.)

Based on lessons learned in the 2009 response, CDC has focused on exploring alternative systems for the distribution and dispensing of antiviral drugs from the SNS. This concept includes leveraging the commercial pharmaceutical distribution chain - drug distributors, pharmacies, etc. - during a pandemic to efficiently move prescription antiviral drugs across the United States to people who need them. This project is still in exploratory stages and is a partnership with CDC, the Association of State and Territorial Health Officials (ASTHO) and the National Association of County & City Health Officials (NACCHO). For additional information please see the following link: <http://www.astho.org/Programs/Infectious-Disease/Antiviral-Distribution/Antiviral-Distribution---Summary-Report/>.

Additional information from ASPR for consideration: This is really more of a CDC-based question. I think the premise is that the issue was with the distribution system, rather than with the initial availability of adequate supplies of the vaccine. The engagement at that time of McKesson, a national distributor for medical supply and equipment, and a participant in the Vaccine for Children program, greatly facilitated the delivery of the available vaccine supply to all fifty states and to multiple addresses identified by the states to the distributor on a daily basis. Certainly, lessons learned during that event highlighted the need for continuous communication with the state and local public health officials who identified the specific localities that were arranged for end-point distribution. Other critical lessons were learned regarding the need for component assembly, so that each vaccine dose has the accompanying ancillary supplies (needles, syringes, alcohol wipes, etc.) as part of a complete, ready-to-go delivery package.

- 2) Given that we have tens of millions of courses of antiviral treatment in the Strategic National Stockpile in advance of a pandemic, it would make sense that some of those reserves could be used to ease shortages in a situation where we are facing severe season flu. Does it make sense to somehow use current or future stockpiled flu treatments during a severe flu season when shortages in the commercial marketplace occur? (Dr Robinson?)

Response (ASPR): The acquisition and distribution of FDA-approved influenza antiviral drugs for pandemic influenza is informed by the PHEMCE, and it is the responsibility of CDC/SNS and state stockpile programs during pandemic periods. CDC has been approached by influenza antiviral drug companies on the concept of using the SNS pandemic influenza antiviral drug stockpile to backfill the market when there are shortages during seasonal influenza outbreaks. Those talks and negotiations are in progress and procurement-sensitive.

- 3) Dr. Robinson: I generally believe that government can provide a higher level of service to constituents by working more like private industry. Are there lessons or best practices we can learn from the private sector when it comes to how we store and distribute countermeasures? In this case, I'm interested in whether the federal government would consider a public-private partnership, where the government owns the medications, but the manufacturers or distributors play a role in the distribution of medical countermeasures during times of need. Has the government explored such an approach, and if not, would you consider it going forward, at least in a pilot project or demonstration basis?

Response (ASPR): HHS has explored and implemented many models for stockpiling and distributing MCMs from industry over the past ten years. These models include the current model (e.g., antibiotics) including vendor-managed inventory (e.g., anti-neutropenia cytokines), federally-subsidized state stockpiles (e.g., influenza antiviral drugs), vendor-stored bulk products (e.g., plasma for botulinum antitoxins), and other novel concepts. Many of these approaches have come from and involve industry. During the 2009 H1N1 influenza pandemic, HHS bought and owned H1N1 vaccines and ancillary supplies from multiple manufacturers via public-private partnerships. HHS distributed the vaccine and ancillary supplies from multiple distribution hubs using a major wholesale pharmaceutical distributor to reach more than 100,000 health care provider sites across the country. SNS uses a third-party logistics model to store and ship MCMs, which has also proven to be a successful public-private partnership.

Pandemic Flu from Industry

- Dr. Robinson, from FY2006-FY2013, pandemic preparedness was largely funded by the FY2006 ~\$6 billion supplemental and remainders from the FY2009 \$8 billion supplemental for the H1N1 pandemic response.
- BARDA's FY2014 budget request indicated "HHS will have spent all of the Pan Flu balances by the end of FY 2013 and will need additional funding to complete the goals defined in the HHS Pandemic Influenza Plan and maintain national preparedness".
- Dr. Robinson : In FY14, the President requested \$140 million in FY2014, of which \$20 million was identified for market activity, e.g., purchase of stockpiles.

- Dr. Robinson: Can you explain the process BARDA uses to determine its allocation of these funds and how incentives to keep the market engaged in these efforts are considered?

Response (ASPR): The National Pandemic Influenza Strategy (2005) and HHS Pandemic Strategic Plan (2005) served as guideposts to HHS agencies to address pandemic preparedness MCM gaps. ASPR/BARDA developed and implemented a comprehensive pandemic influenza MCM plan in 2006 to develop, manufacture, acquire, stockpile, and build manufacturing facilities for vaccines, antiviral drugs, diagnostics, and other medical devices (e.g., next generation portable ventilators). BARDA directed its vaccine development and manufacturing efforts toward expanding domestic pandemic influenza vaccine manufacturing surge capacity and establishing pre-pandemic influenza vaccine stockpiles to meet national pandemic influenza vaccine needs. Similarly, BARDA supported development of new influenza antiviral drugs to treat hospitalized patients with influenza and combat drug resistance. BARDA also built federal and state antiviral drug stockpiles to distribute in an emergency for treatment of patients with influenza.

In addition, with CDC, ASPR/BARDA supported development of rapid point-of-care diagnostics to detect influenza in clinical samples in health care laboratories to inform more reliable and quicker diagnoses and appropriate treatment modalities. Faced with a significant shortage of ventilators in a severe influenza pandemic, BARDA supported the development of new next generation portable ventilators that were less expensive, more user-friendly, equipped with universal components, and designed for adult and neonates.

By investing in the development of innovative and modern MCMs, providing a demand for MCMs through stockpiling in non-pandemic periods to meet emerging novel influenza threats, acquiring MCMs for pandemic events, and cost-sharing in the construction and operation of new manufacturing facilities in the United States, ASPR/BARDA has created solid public-private partnerships that are able to address seasonal and pandemic influenza needs. These partnerships also form a foundation to respond to other emerging infectious diseases. Results from these partnerships are presented below.

From its start in 2006 to now, this HHS plan has delivered on the original goals and in some cases surpassed them. The development approach that BARDA started in 2006 has yielded FDA approval of five first-in-class BARDA-supported MCMs for influenza in the past 18 months. These include cell- and recombinant-based influenza vaccines that modernize the manufacturing process to make more and better vaccine available sooner. Concomitantly, pandemic influenza vaccines with modern adjuvants are available that not only stretch the vaccine supply by using less antigen (i.e., antigen-sparing), but also provide cross-reactive immunity for other influenza virus strains and prolong immunity over multiple years. One antiviral drug candidate, which was used under FDA's Emergency Use Authorization during the H1N1 pandemic, is under review by FDA now. A multiplex rapid diagnostic for point-of-care usage is able to detect several influenza stains simultaneously and respiratory syncytial viruses in clinical samples in 60 minutes with little or no sample preparation. A next generation portable ventilator meeting HHS requirements has been cleared for usage in adults and is under review for usage in neonates.

By 2008, ASPR/BARDA had created and maintained a H5N1 vaccine and adjuvant stockpile to address the emerging H5N1 virus that spread from Asia to Europe in the last decade. BARDA utilized public-private partnerships established with industry through vaccine stockpiling to respond during the H1N1 in 2009 and produce pandemic vaccine. In 2013, we expanded the stockpile following the development, manufacturing, testing, and acquisition of H7N9 vaccine in response to the H7N9 outbreak in China. Federal antiviral drug stockpiles were completed by 2008 and utilized in the H1N1 pandemic. HHS realized 85 percent of the goal for state antiviral drug stockpiles by 2009, and these stockpiles also were used during the H1N1 pandemic.

BARDA increased its public-private partnerships with industry to expand domestic pandemic influenza vaccine manufacturing surge capacity by retrofitting older manufacturing facilities and building state-of-the-art, award-winning new manufacturing facilities. BARDA also established three new flexible manufacturing centers (i.e., the Centers for Innovation in Advanced Development and Manufacturing) and created a network of four fill-finish manufacturing facilities. The result of these efforts is a 4- to 5-fold increase in pandemic influenza vaccine manufacturing capacity now when compared to 2004.

- Dr. Robinson, When will BARDA make available a public version of the 5-year spend plans for its pandemic preparedness programs?

Response (ASPR): This information will be included in the PHEMCE Multiyear Budget that HHS will release this year.

Strategic National Stockpile (SNS)

Last week, Centers for Disease Control and Prevention Director Tom Frieden provided a briefing on this year's influenza activity. It was very helpful when he noted that the first line of defense is vaccination, but, "It's also important to remember that some people who get vaccinated may still get sick, and we need to use our second line of defense against flu: antiviral drugs to treat flu illness."

Our focus on vaccination is appropriate, but we don't talk enough on the importance of treatments for influenza, particularly among children.

According to the CDC, so far this year 52 children have died from influenza, and 43 percent of children who are hospitalized related to influenza had no complicating medical factor. This means that the flu takes a serious toll on our young and vulnerable which why I am troubled about news reports regarding medications that were in short supply in many parts of the country.

We can clearly do a better job of avoiding such shortages by having the government be more flexible on how it approaches the Strategic National Stockpile.

- For example, I understand antivirals purchased for a pandemic are packaged differently than those sold commercially for seasonal flu. This complicates drawing on such a reserve to ease shortages that may occur in severe seasonal flu outbreaks. Has the government thought of working on a system in which the SNS stockpiles antivirals with packaging that could be used for a normal flu season? I am told that in doing so, we could expedite getting medications to the people who need them while also yielding "fresher" with longer expiration dates. What is the

best way to avoid these type of headlines in the future? Are more resources necessary or can this be mitigated by have a better distribution strategy?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

- Because many younger children (and some elderly) are unable to swallow pills or capsules, treatments that come in liquid form, commonly called “suspension.” I am aware the CDC has stockpiled “suspension” formulations of antivirals for pandemic influenza in the past. Can you please tell me how much of this formulation for children is currently in the SNS? How many are we supposed to have?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

- Could we do a better job of easing shortages in the commercial market if the government worked with industry to ensure some consistent level of purchases for the national stockpile, even at a smaller level, thereby keeping production lines open and allowing for production to ramp up quicker as needed?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

Public-Private Distribution innovation

1. I am aware that during the 2009 H1N1 pandemic, the distribution of certain medical countermeasures according to several reports was not as efficient as it could have been. What lessons were learned and what steps have been taken to improve the distribution mechanism during times of need?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

2. Given that we have tens of millions of courses of antiviral treatment in the Strategic National Stockpile in advance of a pandemic, it would make sense that some of those reserves could be used to ease shortages in a situation where we are facing severe season flu. Does it make sense to somehow use current or future stockpiled flu treatments during a severe flu season when shortages in the commercial marketplace occur?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

3. I generally believe that government can provide a higher level of service to constituents by working more like private industry. Are there lessons or best practices we can learn from the private sector when it comes to how we store and distribute countermeasures?

In this case, I’m interested in whether the federal government would consider a public-private partnership, where the government owns the medications, but the manufacturers or distributors play a role in the distribution of medical countermeasures during times of need. Has the government explored such an approach, and if not, would you consider it going forward, at least in a pilot project or demonstration basis?

DUPLICATE QUESTION. SEE PREVIOUS RESPONSE.

BioShield

Dr. Robin Robinson: In last year's (FY 2014) appropriation of \$255 million for the BioShield SRF, the Committee provided funds that exceeded the President's budget request. The 2014 request for the Public Health and Social Services Emergency Fund (PHSSEF) said that the funds would be used to purchase three products – artificial skin replacement, smallpox vaccine for at-risk individuals, and vendor managed inventory for anti-neutropenia cytokines. However, those three items are also listed as planned procurements for the 2015 requested funds. Please explain whether these three products will be procured in fiscal year 2014, 2015 or both years.

Response (ASPR): The FY 2014 appropriation for Project BioShield (\$255 million) is being used to replenish existing stockpiles of expiring anthrax and botulinum antitoxins, smallpox vaccine, and vendor management for the anti-neutropenia cytokines at current preparedness levels. For FY 2015, ASPR/BARDA requests funding to acquire under Project BioShield three new MCMs: (1) artificial skin replacement in radiation-induced thermal burn patients, (2) biodosimetry device and assay to measure radiation exposure more rapidly and with greater accuracy in exposed persons, and (3) a chemical antidote for cyanide poisoning. BARDA also plans to replenish existing stockpiles of expiring anthrax antitoxins and smallpox vaccine at current preparedness levels, and to maintain the vendor-managed inventory of anti-neutropenia cytokines for treatment of radiation patients. The total BARDA request for Project BioShield for FY 2015 is \$415 million. The PHEMCE multiyear budget outlines the most recent spending forecast for HHS' MCM activities, including these planned acquisitions under Project BioShield.

**Questions submitted by
Representative Andy Harris, M.D.**

1) Dr. Korch: The GAO in a report issued in December criticized the PHEMCE for lacking transparency in spending plans (GAO-14-90, December 2013). In the concluding observations GAO stated “HHS would benefit from sharing information on its anticipated spending estimates with industry to assist countermeasure developers with long-term business planning.” Will BARDA provide 5-year spend plans for their pandemic preparedness programs? When will that plan be available? What will BARDA make publicly available to industry and when? Why has BARDA been hesitant to provide this plan?

Response (ASPR): The PHEMCE is working with GAO to identify the specific information that may better address concern regarding transparency in spending plans. We anticipate providing this more specific information in the next version of the PHEMCE Strategy and Implementation Plan.

ASPR/BARDA does not hesitate to provide planning information on its needs to industry in an appropriate manner and at an appropriate time. BARDA has spelled out what its procurement planning looks like for advanced development of products for near-term and long-term needs in publicly provided documents starting in 2007. Providing precise spending plans to the public and our commercial partners is subject to uncertainty in the annual budget process, which is similar to what many other federal agencies, programs, and stakeholders experience due to the appropriations process.

Despite the fact that future years’ annual appropriations cannot be known, the federal government has provided information to biodefense stakeholders that permits such planning to take place. For instance, the now-annual PHEMCE Strategy and Implementation Plan includes a schedule for when various products or approaches will be solicited from industry. This schedule has led to a number of highly successful outcomes in product approvals and product development. The federal government also communicates its business opportunities to industry during events like the BARDA Industry Days and through its Broad Agency Announcements. Furthermore, there are numerous opportunities for novel ideas and product approaches to be considered through the BARDA Tech Watch program, where entities can present a white paper and briefing to officials that may lead to contracts to further develop promising candidates that address government needs.

2) The Department of Homeland Security is obligated to assess what chemical, biological and radiological-nuclear agents pose a material threat against the homeland. There are at least a dozen such threat agents. Dr. Robinson can you tell me for how many of the current threat agents do we have medical countermeasures in the Strategic National Stockpile? How many candidate countermeasures are you currently developing to address material threat agents for which there are no countermeasures in the stockpile? Finally can you tell how many of the material threat agents do we have no candidate countermeasures for? Is it correct to assess that there is a significant amount of effort and work to do to develop additional medical countermeasures to address the current DHS list of material threat agents?

Response (ASPR): The CDC/SNS possesses MCMs for 12 of the 13 threats identified by the Department of Homeland Security. Project BioShield has delivered MCMs for anthrax, botulinum, smallpox, radiological, nuclear, and chemical threats. CDC/SNS has also procured MCMs for radiological, nuclear, and chemical threats, as well as MCMs for anthrax, smallpox and botulism. There also are antibiotics available in the CDC/SNS to treat the medical consequences of other biothreats including plague, tularemia, glanders, melioidosis, and typhus. However, better antibiotics are needed for several of these threats. Currently, there are no licensed or approved therapeutics available yet to treat viral hemorrhagic fever caused by Ebola or Marburg viruses. NIH and DOD are supporting vaccine and antiviral drug candidates that are very early in development. The BARDA CBRN MCM Broad Agency Announcement that was published in July 2013 specifically calls for proposals to support development of MCMs for viral hemorrhagic fever.

ASPR/BARDA has supported development of more than 85 MCM candidates in our development pipeline. Since 2005, we have supported more than 20 vaccine, antitoxin, and antiviral drug candidates for anthrax, smallpox, and botulism—which has resulted in many of them being delivered under Project BioShield and approved by FDA. We are supporting development of eight novel antibiotic drug candidates that offer better treatment of biothreats such as glanders and melioidosis and multi-drug resistant biothreats and public health pathogens. To address the seven subsyndromic illnesses (e.g., hematopoietic, skin and lung trauma, and GI illness) ascribed to Acute Radiation Syndrome (ARS) following exposure to ionizing irradiation, we supported the development of nearly 35 product candidates. For thermal burns, we have supported six candidates. They include artificial skin replacement and antimicrobial ointments. We also supported development of eleven biodosimetry devices and assays to measure radiation exposure. Several new diagnostics candidates are recently supported for biothreat pathogen detection in clinical samples for rapid point-of-care usage. Finally, for chemical threats, we are supporting development of four product candidates for treatment and multiple decontamination modalities.

- 3) Dr. Robinson, last year the President requested only \$250 million for medical countermeasure procurement through the Special Reserve Fund. What types of products do you anticipate being ready for procurement in the next five years? If BARDA were to receive the same level of funding (\$250 million) over the next 5 years would you be able to meet those preparedness goals? If not, what would be a consistent number for each of the next 5 years in order to accomplish these goals?

Response (ASPR): ASPR/BARDA delivered 12 CBRN medical countermeasures to CDC/SNS in the first ten years of Project BioShield and anticipates adding another dozen over the next five years. BARDA is poised to acquire MCMs to treat thermal and radiation burns, biodosimetry and biodiagnostic devices, new antimicrobials, therapies to treat additional subsyndromes of ARS, next-generation anthrax vaccines, and therapies for exposure to chemical agents. In addition, it is also essential that BARDA maintain the manufacturing capabilities established during the first decade of PBS. The upcoming PHEMCE multiyear budget will provide the full five-year spend plan for BARDA.

4) Dr. Robinson, in reviewing recent BioShield procurements and current BARDA advanced development contracts, it appears that HHS has shifted away from developing and procuring vaccines and is investing more in developing and procuring therapeutic products such as antivirals, and antibiotics. Is this a correct assessment of the trend? If so, why was this priority established and is it consistent with the threat?

Response (ASPR): In response to the PHEMCE-developed requirements for the civilian population, vaccines against anthrax and smallpox were key MCMs. However, treatments are the primary MCMs for the other biothreats and the only approach for radiological, nuclear, and chemical threats as described in the PHEMCE Strategy and Implementation Plan (2012). Except for anthrax and smallpox, therapeutic treatments, not vaccines, are the most effective means of ameliorating the medical consequences of these other threats in the civilian population rapidly and effectively.

Vaccination of the civilian population to these threats pre-event, unlike the warfighter, is not an effective strategy nor would it be an ethical approach to address biothreat medical consequences. In the previous decade, anthrax and smallpox vaccines candidates were more prevalent than candidates for other threats. Hence, ASPR/BARDA funded the development of these vaccines. However, BARDA also supported development of treatment modalities such as antitoxins and antiviral drugs for anthrax and smallpox during this time. Today, BARDA continues to support advanced development of these anthrax and smallpox MCMs while balancing support for newer therapeutics and therapies for the other threats.

HHS has made significant progress against the two primary threats for which vaccines would be efficacious: smallpox and anthrax. HHS maintains enough smallpox vaccine for the entire U.S. population for general use as well as a smallpox vaccine (MVA) for special populations (e.g., individuals with HIV infection or atopic dermatitis [AD]). The MVA vaccine can be used during an emergency in individuals with either HIV or AD, including people of all ages and pregnant and nursing mothers. BARDA and CDC have also procured anthrax vaccine to be used during an emergency for post-exposure prophylaxis (PEP). BARDA continues to support this product for licensure of PEP and has several next-generation anthrax vaccine programs under ARD. BARDA is also considering new programs to support vaccines for the threat of viral hemorrhagic fever. Additional vaccines for CBRN threats are not currently under consideration because the primary role for response would be in a post-exposure setting where therapeutics would be needed.

5) Mr. Burel, once a medical countermeasure is developed at BARDA, the CDC plays an important national security role in managing and distributing emergency medical supplies in the Strategic National Stockpile. How does CDC work with BARDA to ensure that stockpiles of new medical countermeasures initially procured through BARDA/ Special Reserve Fund (SRF) are maintained and replaced prior to and post licensure? At what point does stockpile procurement and maintenance responsibility transfer from BARDA to CDC?

Response (CDC): Project BioShield funding under the Special Reserve Fund (SRF) does not usually support maintenance costs of FDA-approved or –licensed medical countermeasures

(MCM) procured using funds from the SRF and added to the SNS, or the replacement of those MCMs that have been transferred to CDC-funded contracts once they are commercially available products. Beyond FY 2015, budget requirements for SNS may be significantly impacted by increased procurements using the SRF, either through the transition of replacement responsibilities to CDC, or through expanded storage requirements for new product lines.

In consultation with PHEMCE, SNS is reducing procurement of some expiring countermeasures in order to maintain capability for other MCM. These trade-offs are necessary to balance the PHEMCE priorities with available financial resources.

6) Dr. Robinson, from FY06-FY13, pandemic preparedness was largely funded by the FY06 ~\$6 billion supplemental and remainders from the FY09 \$8 billion supplemental for the H1N1 pandemic response. BARDA's FY14 budget justification indicated "HHS will have spent all of the Pan Flu balances by the end of FY13 and will need additional funding to complete the goals defined in the HHS Pandemic Influenza Plan and maintain national preparedness." In FY14, the President requested only \$140 million (less than 1% of previous investments) for all R&D and acquisition for the entire portfolio of vaccines, antivirals, diagnostics, ventilators, etc. Only \$20 million of the \$140 million request was identified as the market, e.g., purchase of stockpiles. What incentives does BARDA now offer private sector partners given the virtual elimination of the market and lack of transparency with future spend plans? Is FY14 funding (\$115 million) sufficient to complete the goals and maintain national preparedness? Is that consistent with the funding targets identified in the 2010 PCAST report on the Pandemic Influenza?

Response (ASPR): ASPR/BARDA is continuing to support and prioritize development stockpiling, manufacturing, and facility construction of MCMs for pandemic influenza using remaining supplemental appropriations and FY 2014 appropriations. This support is described in BARDA's Broad Agency Announcement (2013) and was discussed with industry stakeholders at BARDA's Industry Day (2013). Furthermore, the President's FY 2015 Budget request highlights and provides funding for new pandemic influenza preparedness initiatives to develop universal influenza vaccines and new immunotherapies for treating influenza infections. BARDA's multiyear budget for FYs 2014-2018 accommodates the completion of existing vaccine and antiviral development programs that have already borne new FDA-licensed products, maintenance of pre-pandemic vaccine stockpiles, completion of construction at new domestic vaccine manufacturing facilities (e.g., the Centers for Innovation in Advanced Development and Manufacturing), and the launch of the aforementioned new vaccine and antiviral therapy initiatives that may transform seasonal and pandemic influenza preparedness. Previous, current, and future investments in pandemic preparedness address the recommendations provided in the report of the President's Council of Advisors on Science and Technology (2010) and have already delivered a modern and flexible influenza vaccine manufacturing capacity in the U.S. that is able to make more and better vaccine available sooner.

7) Dr. Robinson, your February 11, 2014, briefing to NVAC provided information on pre-pandemic stockpiles of H5N1 antigen and adjuvants purchased mostly in 2005-2009. These stockpiles are now approaching shelf lives of 5-10 years. How long do both the antigen and adjuvant stockpiled by HHS last? I understand that H7N9 will require two doses of adjuvanted vaccine. Do you think that the current H7N9 stockpile of 30-38M doses is sufficient to adequately protect the public given that two doses are needed? Is the amount of adjuvant in the stockpile sufficient if an outbreak occurs that may require an adjuvanted vaccine?"

Response (ASPR): The HHS stockpile of H5N1 antigen and adjuvant are stored in a bulk format that has proven to be able to maintain remarkable stability over time, as determined by validated potency and stability indicating assays and measured on a regular schedule by each manufacturer of these stored products. ASPR/BARDA has replenished the antigen stockpile as warranted and will continue to monitor both antigen and adjuvant stockpiles for potency and applicability to emerging novel influenza strains with pandemic potential through the annual HHS influenza risk assessment.

Preliminary clinical data evaluated to date have indicated that it would likely require two doses of adjuvanted H7N9 vaccine to provide sufficient immunity in an individual. By making the H7N9 vaccine, manufacturers have gained valuable experience in producing a new vaccine against a novel influenza virus that has proved difficult in the past and was previously shown to have been poorly immunogenic. This experience buys valuable time in the ability to respond to a widespread outbreak of H7N9. The clinical experience gained has provided valuable data on the safety, dosage, and immunogenicity of the H7N9 vaccine, which also saves valuable time in a response. In addition, we have stockpiled H7N9 vaccine and adjuvant that could be used to protect the critical work force, consistent with the National Strategy for Pandemic Influenza. Both H5N1 and H7N9 vaccine stockpiles also provide seed strains that are optimized for further vaccine production in the event that these threats become pandemics. The adjuvant stockpile is sufficient to cover the H7N9 vaccine stockpile.

8) Dr. Robinson: A primary vaccine goal in the National Pandemic Influenza Strategy is to "Establish and maintain pre-pandemic vaccine stockpiles to immunize 20M people against influenza strains that present a pandemic threat". How is BARDA progressing toward that goal for H7N9? What investments were made and what lessons is BARDA learning from the H7N9 pandemic threat? What are the funding needs to rapidly and effectively respond to pre-pandemic threats? Does BARDA have an effective operational model and investment strategy for pre-pandemic and other emerging threats?

Response (ASPR): With HHS interagency partners at NIH, CDC, and FDA, ASPR/BARDA was able to develop, manufacture, clinically test, and stockpile H7N9 vaccines in 2013 at a cost of approximately \$150 million in response to the H7N9 outbreaks in China. The five vaccine candidates supported by this effort showed immunity consistent with protection following two doses. The H7N9 vaccine stockpile is sufficient to address the vaccination needs of the critical work force that would maintain the continuity of government and societal stability as intended in the National Strategy for Pandemic Influenza (2005).

The progress made in influenza vaccines through the Influenza Vaccine Manufacturing Improvements initiative recommended in the MCM Review (2010) and PCAST report (2010) were applied successfully in the development and manufacturing of the H7N9 vaccines. These improvements included a shorter time to prepare vaccine seed strains using synthetic biology approaches, quicker optimization of vaccine production, new alternative potency assays that were comparable to classic methods and not reliant on potency assays reagents (which take 4-8 weeks to prepare), and faster and more sensitive sterility assays for vaccine product release. Obstacles in virus and vaccine seed transport and biosafety permitting were identified and resolved during the H7N9 response. The 1 stockpiling estimates will be included in the 5-year Budget.

BARDA's investment strategy has delivered to date three new and modern FDA-licensed vaccines (cell- and recombinant-based, antigen-sparing vaccines) in the last 18 months, with more to come this decade. BARDA also has established robust and sustainable H5N1 and H7N9 vaccine and adjuvant stockpiles and built a U.S. pandemic influenza vaccine manufacturing surge capacity that is 4-5 fold greater than the capacity that was available in 2004. Investments in universal influenza vaccine development by BARDA with NIH through the rest of this decade may transform both seasonal and pandemic influenza preparedness to make more effective vaccines available with long-lasting immunity against most influenza viruses.

9) Dr. Robinson: I understand there are now over 80 products in development at BARDA. This clearly shows the success over the last few years in getting more companies to partner with the United States government. Given that the Pandemic and All-Hazards Preparedness Reauthorization Act (PAHPRA) allows for procurement contracts up to 10 years before FDA approval, how many products in the BARDA pipeline should be moving to BioShield contracts over the next 1-3 years? Are there requirements for procurement?

Response (ASPR): Consistent with the FYs 2014-2018 multiyear budget under Project BioShield, ASPR/BARDA expects that 12 CBRN MCM candidates will mature sufficiently to afford transition from BARDA ARD to acquisition under Project BioShield. All of these new MCMs are covered by existing Material Threat Assessments from the Department of Homeland Security and MCM requirements from the PHEMCE.

10) Last August, Syria used Chemical Weapons against its own people. Even before that, US and allied military personnel carry medical kits as part of their equipment which contain both nerve agent antidotes and the FDA cleared rapid skin decontamination lotion (RSDL) that can rapidly remove and neutralize a broad array of other WMD chemical threat agents including mustard, sarin, soman, VX nerve agents, and many others. Are HHS and CDC going to include the RSDL packs in their CHEMPACK program so that emergency responders and others can be protected against this broader array of chemical threats in addition to nerve agents?

Response (CDC): The PHEMCE Chemical Integrated Program Team (IPT) has not recommended decontamination supplies, including RSDL, and, therefore, there is not a current

requirement for inclusion in CHEMPACK containers. It is our understanding that the IPT recognizes the value of RSDL as an effective product for spot decontaminations, but the IPT finds soap and water or just water to be an effective and less costly alternative.

Decontamination, while a critical function of the response to a nerve agent or other chemical release, does not rely on the type of costly or rare medical countermeasure that must be stockpiled by the federal government to ensure availability and access at the time of need. The equipment required is bulky and relatively inexpensive for local procurement, and the most cost effective agents for mass decontamination are soap and water. None of these supplies would be appropriate or effective for federal procurement and storage in CHEMPACK caches.

11) BARDA's recent broad agency announcement includes topics for the development of next generation decontamination products. Given that RSDL is FDA approved and currently used by DoD, what measures is the Public Health Enterprise taking to ensure availability of the existing product to all who can benefit from it, while also seeking improvements through the BAA process?

Response (ASPR): The PHEMCE's Chemical Threat Integrated Program Team is deliberating about rapid skin decontamination lotion (RSDL)-type products. The team is considering such things as quantities, deployment mechanisms, and concept(s) of operations as part of the recurring SNS annual review process. ASPR/BARDA is continuing to fund decontamination studies and products for chemical agents and welcomes proposals for improved decontamination modalities and products through our current Broad Agency Announcement on MCM development for chemical agents.

12) The committee is pleased to know that CDC has acquired a significant stockpile of FDA approved anthrax vaccine. What efforts, if any, are being made to manage the lifecycle of that product, to include perhaps providing access to state and local first responders to product that may be about to expire?

Response (CDC): The use of FDA approved anthrax vaccine by the Department of Defense and state and local first responders is a consideration made independent of the expiration of the product. CDC maintains a joint stockpile of Anthrax and Smallpox vaccines for the SNS and DoD. Under this arrangement, CDC maintains the vaccine in SNS facilities with a reserve amount set aside for DoD use. As product is required for their vaccination programs, DoD requests shipment of specific quantities from the SNS. CDC is reimbursed for the amount of vaccine delivered to DoD and is able to purchase new vaccine to restore on hand balances.

HHS, as represented by CDC and ASPR, is currently working with the Department of Homeland Security's Office of Health Affairs is examining the demand for and feasibility of providing

anthrax vaccine from the strategic national stockpile to communities for the vaccination of their responders.

Vaccination of responders may improve response capability to a wide-area anthrax attack by having vaccinated responders, able to deploy immediately and confident that they have been afforded as much protective status as possible for these activities. Vaccination may also offer greater protection if responders are exposed to a drug resistant strain of anthrax or overwhelming number of spores. CDC has committed to provide stockpiled anthrax vaccine from the SNS to support this new pilot, and continues to support the interagency effort to implement a safe and effective program to accomplish this goal.

13) Dr Robinson: What is the regulatory and development status for next generation recombinant rPA anthrax vaccines and what are the Department's plans for continued investment in these investigational vaccine candidates?

Response (ASPR): ASPR/BARDA funded four projects supporting development of recombinant protective antigen (rPA)-based anthrax vaccines. One project was recently down-selected because of issues with stability of the product and the FDA's placing the program on clinical hold twice within two years. Two of the three remaining rPA anthrax vaccine candidates are developing lyophilized formulations that have shown increased stability. In addition, one of the developers is utilizing a version of the protective antigen gene that produces a protein that has mutated cleavage sites, thereby increasing the stability of the molecule. Both approaches have shown great promise in achieving stability of the molecule. Both of these candidates are at the pre-Investigational New Drug stage, and BARDA will continue to fund the programs as long as the developers meet scientific milestones. The third candidate utilizes an adenovirus vector to introduce the anthrax Protective Antigen gene in a single-dose, intranasal administration. This approach eliminates the rPA stability issue. Animal challenge studies with this candidate showed good protection against inhalational anthrax, and this candidate is now under development for clinical trials.

**Questions submitted by
Representative ROSA L. DELAURO**

In carrying out its mission, BARDA provides substantial assistance—both financial and otherwise to private companies for the development of drugs, vaccines and other products.

Dr Robinson: Please tell us what steps BARDA takes to try to protect the public interest arising from this investment. Certainly, much of what the public gets in return is development of medical countermeasures that could be of tremendous benefit in the event of a terrorist attack but for which there is insufficient private market demand to support development on a purely commercial basis. But I'm interested in knowing what specific steps BARDA takes to try to make sure that the benefits of the work it supports go to taxpayers as well as the private companies being assisted.

- For example, are there requirements that data and technology arising from these efforts be made available for use by the government or other contractors, at least under some circumstances?

Response (ASPR): ASPR/BARDA is committed to sharing non-proprietary and non-procurement sensitive information on MCMs with other U.S. government agencies, the private sector, and the academic community. In addition to the usual methods such as publication of results in scientific peer-reviewed journals, BARDA has provided opportunities for U.S. government and industry study results to be shared in a pre-competitive space. For example, results of animal challenge studies with CBRN agents from government laboratories, U.S. government-sponsored laboratories, and industry affiliated with BARDA's Non-Clinical Animal Study Network are shared among interested U.S. government and industry partners to facilitate the understanding and utilization of qualified animal models for development of MCMs. Patentable inventions by companies that emanate from U.S. government support are transferred to other companies to produce MCMs in an emergency. BARDA served as a conduit for the transfer of reverse genetics technology from a company and several academic institutions to vaccine manufacturers for the development and manufacturing of H5N1 and H7N9 vaccines to build U.S. government pre-pandemic influenza vaccine stockpiles and the development and manufacture of H1N1 vaccines during the 2009 pandemic.

Another way that BARDA has made study results available to the private sector and the larger scientific community is by reaching agreements among vaccine manufacturers, U.S. government laboratories and agencies, and academic institutions in the HHS Influenza Vaccine Manufacturing Improvement initiative to share the results of studies to optimize influenza vaccine seed strains for greater vaccine production. All parties agreed to develop and share data without proprietary ownership for the better good of the industry and research field. Some of these results enhanced our ability in 2013 to prepare vaccine seed stocks rapidly for H7N9 vaccine production at high yield. Finally, BARDA routinely shares non-proprietary information at meetings through formal presentations or posters.

Where the USG has rights to patentable inventions including platform technologies and manufacturing processes for medical countermeasures (e.g., antibiotics), BARDA like other

federal agencies may exercise its licensing rights by making the intellectual property (IP) available to a third party to utilize the IP to perform services or produce products for the USG. IP that the USG does not have rights may be licensed by the USG from the IP holder and utilized for USG needs according to the terms of the licensing agreement.

Are there requirements that, if successful, the company stay in the business of producing the countermeasure for some time, or transfer the technology and licenses to another potential manufacturer?

Response (ASPR): ASPR/BARDA has no requirement for a business developing and manufacturing to stay in business. However, we have long-term contracts (i.e., up to 25 years) with companies to produce MCMs as needed. The public-private partnership between BARDA and Novartis maintains a long-term relationship for their new cell-based influenza vaccine manufacturing facility in North Carolina to produce vaccine each year and during public health emergencies. The Centers for Innovation in Advanced Development and Manufacturing (CIADM) are further examples of these long-term partnerships between BARDA and vaccine manufacturers and academic institutions to develop MCMs routinely but provide massive manufacturing surge capacity in a public health emergency. If a company is not sustainable for whatever reason, there is the potential for the manufacturing process to be transferred to the newly established CIADM to maintain the availability of the product for the federal government.

BARDA routinely monitors the business transactions of MCM developers and manufacturers. We provide input to federal regulators on the sustainability of MCMs and national security impacts, when companies manufacturing MCMs are sold to other companies (e.g., MedImmune to Astra Zeneca in 2007; Human Genome Sciences to GlaxoSmithKline in 2012).

Strategically, BARDA has positioned its investments since 2011 towards existing products that may be repurposed from previous commercial indications to CBRN indications and new product candidates that have both CBRN and commercial indications to afford greater MCM and company sustainability. BARDA's entire broad spectrum antimicrobial program has been built since 2010 on this strategic principle, resulting in the awakening of a dormant antibiotic drug development industry sector and eight novel antibiotic candidates in our ARD pipeline to treat biothreats and public health pathogens including multidrug resistant diseases such as MRSA and CRE. BARDA has utilized multiple approaches successfully to support greater MCM business sustainability and will look for more effective and creative ways going forward to keep the nation prepared for emergencies.

- Do your advanced development contracts address in any way the price at which an eventual product would be sold to the government?

Response (ASPR): As a good steward of federal government dollars, ASPR/BARDA is always looking for ways to decrease the cost of MCMs to the U.S. government and for the American consumer. The eventual cost of the product is a major consideration for BARDA in the development, manufacturing, and acquisition of MCMs. BARDA manifests this commitment in numerous ways during product development. They include seeking to reduce the number of

doses for a treatment or vaccine regimen, expanding product shelf life, removing cold storage requirements for products, creating company competition with multiple players, and implementing vendor-managed inventory strategies.

BARDA requires MCM developers to submit project plans that include potential product costs based on the costs of good to manufacture. We also compare these prices with similar industry products as benchmarks to determine comparability. BARDA evaluates these data as part of the selection process and calculates the overall life-cycle management costs across the U.S. government to acquire and replenish those candidates selected by BARDA for investment into product development. The PHEMCE and ASPR/BARDA multiyear budgets reflect these life-cycle cost estimates.

- In the event that a development effort is not successful, are there provisions for making use of the research in other BARDA projects?

Response (ASPR): ASPR/BARDA supports development of medical countermeasures that may result in patentable inventions for methods, assays, and platform technologies. The USG has non-exclusive licensing rights to intellectual property (IP) derived from USG support and may utilize that IP for its own purposes or transfer to a third party to provide services or produce products for the USG using that IP. For IP that the USG does not have licensing rights, the USG may license the IP from the IP holder for USG needs according to the terms of the negotiated licensing agreement.

In addition, pilot and commercial-scale products that are manufactured with BARDA's funding are owned by the U.S. government and have been shared with other government agencies under a material transfer agreement for further studies and with other manufacturers for the benefit of the U.S. government. If a development effort is not successful, it may be necessary for the U.S. government to sign a licensing agreement with the company to gain access to the company's IP that is not freely accessible to the U.S. government.

- In the event that those efforts lead to a product with a strong commercial market, do BARDA contracts make any provision for sharing the gains between the government and the public? I realize that most potential products will not have much of a private market, but there could be exceptions, such as with the efforts to develop broad spectrum antibiotics and antivirals.

Response (ASPR): Presently, ASPR/BARDA does not have contractual or other agreements that afford a return on investment as capital investors enjoy on their investments. The Administration has proposed a Strategic Investor (SI) that would provide an equity-position in companies for ASPR/BARDA's investments in the development of MCMs with commercial markets (i.e., antibiotics, oncology drugs). BARDA would derive monetary benefits from successful commercialization of these products and re-invest these proceeds back into the SI. BARDA continues to seek authorization and appropriations to launch the SI. The President's FY 2015 Budget request proposes to authorize and provide \$20 million for SI.

Dr. Robinson: BARDA is able to enter into incrementally funded, multiyear contracts for up to 10 years rather than the standard limit of 5 years. The multiyear contracting authority provides industry with a clear indicator of sustained, critical support from the federal government. However, funding for BARDA is now provided through annual budget authority rather than through transfers from the Project BioShield Special Reserve Fund advance appropriation. In your opinion, have any unintended consequences surfaced, positive or negative, from moving from a sustained availability funding stream to annual appropriations and/or giving BARDA the authority to enter into longer contracts?

Response (ASPR): ASPR/BARDA has not been affected adversely in the transition from advanced and supplemental appropriations to annual appropriations. We are grateful for Congress' support in providing appropriations for FY 2014 consistent with the President's budget. We also appreciate enactment of the language the Administration requested to enhance our ability to enter into longer contract term lengths and look forward to using it when appropriate.

**Questions submitted by
Congressman Mike Honda**

- 1) There has been no recent change in the material threat determination give to this committee, and there are existing pre-event countermeasures that address the anthrax, smallpox, radiation, and nerve agent threats to first responders. Some of these have yet to be fully fielded or funded. What plans and budget requests have been included by the Department to protect our protectors at the local and state levels? Are those plans the same as plans for the federal workforce?

Response (ASPR): First responders are critical in supporting and assisting communities as they recover from the impacts of public health and medical incidents. Federal responders receive MCMs from CDC/SNS. The distribution locations are co-located with mobilization processing sites.

Planning considerations for the protection of first responders is primarily done at the state and local levels. Federal agencies provide general recommendations, modeling projections, and other supporting information to guide such planning efforts. Recently, a federal interagency working group that included subject matter experts from DHS and HHS issued its “Guidance for Protecting Responders’ Health during the First Week Following a Wide-Area Aerosol Anthrax Attack.” This non-binding guidance provides recommendations to protect first responders and addresses pre- and post-event vaccination, use of personal protective equipment, and personal decontamination processes.

In addition, HHS and DHS recently collaborated on a letter to first responder occupational health providers to promote the implementation of a MedKit program to provide MCMs to first responder personnel prior to a biological event. In this correspondence, federal officials outlined the benefits of such a program and suggested how it could be implemented with individual first responders’ contacting their personal health care providers to obtain prescriptions for recommended antimicrobials. The cost of the MCMs would be borne by the individual first responder, if not by the state or local responder organization.

Response (CDC): CDC supports activities to address the needs of all state and local first responders to minimize risk and prepare to do their job under the most demanding circumstances. The CHEMPACK program is one CDC activity which directly impacts responder safety. CHEMPACK caches are forward deployed containers of CDC owned nerve agent antidote, held in local custody which allows for immediate use by state and local first responders or healthcare staff in a chemical nerve agent response without any requirement for federal decision-making or deployment.

Additionally, HHS, as represented by CDC and ASPR, is currently working with the Department of Homeland Security’s Office of Health Affairs to examine the demand for and feasibility of providing anthrax vaccine from the strategic national stockpile to communities for the vaccination of their responders.

CDC has committed to provide stockpiled anthrax vaccine from the SNS to support this program, and continues to support the interagency effort to implement a safe and effective program to accomplish this goal.

- 2) Given the unique responsibilities of emergency services and health care workers continue to operate in face of outbreaks and terrorist attacks does the Department have a specific budget plan for protecting this workforce and their households? How can the PHEMCE review be expanded to include this operational necessity?

Response (ASPR): Health care workers and first responders are critical in mitigating the lasting impacts of a wide-spread disease outbreak or terrorist attack. Health care workers follow universal precautions daily. These precautions may be updated, and other precautions may be added during an outbreak or terrorist attack depending on the specific characteristics of the agent. CDC issues guidance and recommendations on precautionary practices and encourages all health care organizations to adopt such practices in their daily infection control and emergency response plans. Accreditation organizations have adopted these standards and require adherence in order to obtain and maintain accreditation. Specific to a pandemic influenza outbreak, state and local authorities will receive products from the CDC/SNS including antivirals, vaccines, and other MCM and mitigation materials (ventilators, masks, etc.). State and local authorities would distribute products in accordance with need and supporting planning documents.

The 2012 PHEMCE Strategy and Implementation Plan (SIP) has an objective and implementing language to develop and communicate MCM utilization policy, guidance, and response that are responsive to end-user needs and that are integrated with state, local, tribal, territorial, and private-sector response plans. CDC leads these efforts, generally through the Division of State and Local Readiness. In addition, specific deliverables identified in the PHEMCE SIP include the development of response strategies, MCM dispensing Concepts of Operation, medical utilization guidance for specific products, and clinical practice guidelines to ensure that the information in MCM response integration and utilization plans will be readily accessible in an emergency.

Response (CDC): CDC directly supports the needs of the healthcare and emergency services populations who form the front line of public health response in emergencies through the activities of the Healthcare Preparedness Activity (HPA) within CDC's Strategic National Stockpile. HPA works to improve the healthcare delivery system's ability to respond to potential natural disasters and public health threats, such as pandemic influenza.

EMS plays a vital role in response to public health threats including transport to alternate sites, treatment/referral without transport, alternate dispatch, and distribution of non-vaccine countermeasures. Recognizing this vital role, CDC, in collaboration with the Oak Ridge Institute for Science and Education (ORISE) and the U.S. Department of Transportation's (DOT) National Highway Traffic Safety Administration Office, is developing tools to assist state and local jurisdictions with developing and coordinating expanded EMS roles during medical surge events.

- 3) The Chempack program has not had a full review since its inception more than 10 years ago. In that time we have become less protected, with fewer caches forward deployed. Local and state program managers have initiated a dialog and suggested areas for program enhancement. Is the Administration considering a funding request to undertake this review with the local program managers?

Response (CDC): CHEMPACK caches are forward deployed containers of CDC owned nerve agent antidote, held in local custody which allows for immediate use by first responders or healthcare staff without any requirement for federal decision-making or deployment.

CDC works closely with state, local and territorial partners to sustain and relocate containers as necessary to meet local response plans and requirements, and support temporary container movements for operational concerns or special event coverage. As of October 2011, all 62 PHEP awardees (including the 50 states, 4 directly funded cities, and 8 U.S. territories and freely associated states) are participating in CHEMPACK with 1,961 CHEMPACK containers held in more than 1,300 state and local storage sites across the nation.

As with other SNS products and capabilities, the CHEMPACK program is reviewed annually through the PHEMCE process with results and guidance for adjustments to CHEMPACK communicated through the mandatory SNS Annual Review. New formulary changes were recommended in the 2012 SNS annual review including replacement of the existing anticonvulsant with a new anticonvulsant currently under contract by BARDA. These changes will take effect as soon as the product is available.

In addition to continuous contact with state and local officials in the process of sustaining fielded CHEMPACKs, CDC has engaged with the Association of State and Territorial Health Officials (ASTHO) and the National Association of City and County Health Officials (NACCHO) to understand the recommendations and requirements that could continue to improve CHEMPACK. NACCHO has formed a workgroup to study this issue to advance the CHEMPACK program.

As in other aspects of the SNS, our direct engagement with state and local officials and other public health partners guides our work to maximize the response capabilities realized through investment in this program. As an example of these partnerships, CDC is working with New York City on a design of CHEMPACK containers that will better suit the specific needs of the city's first responders.

THURSDAY, MARCH 13, 2014.

U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES

WITNESS

HON. KATHLEEN SEBELIUS, SECRETARY

Mr. KINGSTON. Well, we will go ahead and call the committee to order.

And, Madam Secretary, I welcome you to the Subcommittee on Labor, Health, and Human Services.

And I will go ahead and have my opening statement. And I am sure that, Rosa, you will have one, as well.

You are center of so many things that are going on right now in Washington that we are going to have a lot of questions for you.

But I wanted to, first of all, remind everyone on the committee that we will do the 5-minute rule, as we always have. And we will go in order of appearance, if somebody comes in after this.

But, Madam Secretary, we are pretty strict on the 5 minutes. It applies to witnesses and Members.

I do have a lot of concerns in your budget. And, last year, you had estimated in our questions that the cost of implementing the Affordable Care Act was between \$5 billion and \$10 billion. And that was, as I said, part of our Congressional Record. That is a big swing, but it is still an underestimation.

If you look at this year's request, there is an assumption for \$430-million brand-new mandatory program used for CMS program management, and then you assume \$1.2 billion next year for userfees and then \$639 million in annually appropriated discretionary dollars for the exchanges. That totals to \$2.2 billion, which is a 41-percent increase over last year, or \$643 million.

So I am concerned about that. But then when we read further in your budget, if you add in approximately 21 billion other dollars that are out there for State exchange grants, consumer operated/oriented plan, preexisting condition insurance plan, early retirement reinsurance, transitional reinsurance, risk adjustment program, and risk corridors, if you add those two together, it is \$23 billion to run Obamacare.

Right now, you have 4.2 million people who are in line to enroll. Some of them are enrolled, but not all of them are enrolled right now. Your estimation is that 8 million people will be enrolled next year. If you look at that 8 million people, compare it to the 23, it comes out to nearly \$3,000 per enrollee.

Now, the President said repeatedly—repeatedly—that the average person would have a \$2,500 premium decrease. But just for the government to get involved, it is \$3,000 per enrollee. So not only has that decrease not happened, but you have this huge government expansion and burden. Because in addition to that \$3,000,

the insurance companies, of course, have an overhead charge of their own.

So when we talk about this new law and the cost of it, to me, that is the most disturbing of anything.

I will say, in my discussions with constituents, I have not found anybody that has had a premium decrease. I have not talked to many businesses who have created more jobs or found Obamacare compliance business-friendly in terms of creating more jobs. So, to me, it is a failure.

It was supposed to decrease premiums and increase access, but when businesses are not creating jobs, or putting people on a part-time basis in order to get around Obamacare, or keeping the number of employees below 50, then it has been a failure on both sides of it, the premium side and the access side.

So those are my concerns, and we will look forward to having good exchanges on it.

And let me yield to my friend, the ranking member, Ms. DeLauro.

Ms. DELAURO. Thank you very much, Mr. Chairman.

And welcome, Madam Secretary. Thank you very much for joining us. Thank you for all that you have done and are continuing to do to implement the Affordable Care Act, which, in fact, is a transformative law for American families.

Before I begin, I want to make three important points to help guide our conversation this morning and to set the record straight.

First, and despite what we are likely to hear, Congress has spent the last few years making deep and irresponsible cuts to non-defense discretionary spending. If history is any guide, the commentary will be to suggest that spending on these vital programs has grown or even exploded in the past decade. This is simply not true.

Let us look at the evidence. A common means of comparing budget levels over time is to measure them relative to the size of the economy as a percentage of the gross domestic product. Using that measure, per capita inflation-adjusted spending on programs funded in the Labor-HHS bill over the past decade has been cut by nearly 15 percent, from 1.2 percent of GDP 10 years ago to just 0.95 percent in the 2014 budget we enacted 2 months ago.

NIH has been cut by almost \$1.1 billion; HRSA by \$1.4 billion; CDC by \$723 million. Job training programs at the Department of Labor have been cut by \$696 million; Title I by \$107 million; IDEA by \$32 million. The list goes on and on.

Some of my colleagues may applaud these deep cuts as a necessary austerity, but in real-life terms what we are really talking about here is less money for education, less money for scientific research, less money for public health investments, among other critical priorities across the Labor-HHS bill. We are not doing more with less; we are doing less with less.

We are still working to recover from the worst recession in generations, and yet we are shortchanging the critical investments in our future that actually grow the economy and save money in the long run. The recent budget agreement was a small step in the right direction in that it reversed some, and some only, of the deep and indiscriminate sequester cuts. But we still have a long way to

go. For example, the 2014 budget restored only 58 percent of the sequestration for NIH, a critical driver of jobs in health. CDC remains \$100 million below its funding level prior to the onset of sequestration, despite the continued emergence of public health threats. Just two examples of where we fell short. There are many more.

Second, I want to highlight the success of the Affordable Care Act. We all know there were serious problems with the initial roll-out of HealthCare.gov last fall, and I expect we will spend time this morning discussing what went wrong there. But I also want to make sure we acknowledge and that we applaud the many policy successes we have seen so far.

Since the HealthCare.gov Web site fixes went live, enrollment numbers have shot up nationally. Over 13 million Americans have signed up for affordable insurance coverage, many for the first time. In my State, AccessHealthCT, the State-run exchange, had a goal of enrolling 100,000 people by March 1st. It has enrolled close to 160,000 Connecticut citizens. It is coming in on time and under budget.

A new Gallup poll shows that since the Affordable Care Act went into effect the uninsured rate in America is dropping among every single demographic group, especially low-income Americans. Healthcare spending growth is the lowest on record. In fact, healthcare spending growth rates over the past few years are less than one-third of the long-term historical average going back more than 50 years.

Due to the slower growth in healthcare spending, CBO projects that the Affordable Care Act will reduce Federal deficits by \$100 billion in the next 10 years and by an average of \$83 billion per year in the subsequent decade. I repeat, CBO: Affordable Care Act will reduce the Federal deficit by \$100 billion in the next 10 years and an average of \$83 billion per year in the subsequent decade.

So, notwithstanding the rhetoric, evidence so far suggests that the Affordable Care Act is working and it is providing more Americans access to affordable insurance, a higher quality of care, while working to slow the growth of healthcare spending and healthcare inflation.

Let us not lose the forest for the trees. Americans do not want us to repeal the Affordable Care Act. They want us to fix what is not working as well as intended and to move forward.

Third, I want to turn to the main question before us today, the Health and Human Services budget request for fiscal year 2015. I was pleased to see modest increases for critical programs and priorities like biomedical research and early childhood programs in the President's request. At the same time, other parts of this request give me serious pause. For example, the proposal would further reduce the Low-Income Home Energy Assistance Program, or LIHEAP, by another \$625 million. Right now, LIHEAP's current funding is still below the pre-sequester level. I am also troubled by the proposal to cut community services programs by nearly one-half. And I hope we can talk about these priorities.

Finally, I have a question for my fellow members of the subcommittee, particularly those who are concerned about waste, fraud, and abuse in health care: Why did we choose not to fully

fund the Health Care Fraud and Abuse Control Program in the 2014 budget?

This program acts as a deterrent against fraud and overpayments in our Medicare system. It saves billions of dollars of taxpayer money. It ensures that our seniors receive the benefits that they have earned. And yet the majority left an additional \$329 million for this program on the table, even though it would not have cost this committee a penny from other programs due to the cap adjustment for program integrity initiatives. These additional funds would have saved taxpayers approximately \$2.5 billion if we had included them in the recent budget.

If we are concerned, and truly concerned, about stopping healthcare fraud, reducing the deficit, we need to fund the programs that work to do so. I hope my colleagues will commit to fully fund this program for 2015.

So we have much to talk about. And with that in mind, Secretary Sebelius, I thank you for coming today, for your hard work on behalf of our families. It is a tough job. I look forward to hearing your testimony and for the discussion. Many thanks.

Thank you, Mr. Chairman.

Mr. KINGSTON. Thank you, Ms. DeLauro.

Perhaps we could find some compromise. We could take the money out of Obamacare and put it into fraud. What do you think? I don't think you like that—

Ms. DELAURO. You need to stop repealing—trying to repeal the bill. Fifty-one times now, I think, you know? They say “insanity” is repetition over and over and over again.

Mr. KINGSTON. You mean like spending more money on big government for solutions?

All right. As you can tell, Ms. Secretary, my good friend and I may have a slightly different view of this, but we share a lot of views in common on other issues. And we are looking forward to your testimony.

So, with that, I will yield the floor to you. And, again, 5 minutes, so you may need to skip around, but we have your written testimony. Thank you.

OPENING STATEMENT

Secretary SEBELIUS. Well, good morning, Chairman Kingston and Ranking Member DeLauro, members of the committee. I am pleased to be here again.

As President Obama has said, the budget you consider is about more than numbers. It is about our values and what sort of future we want to give to our children. Among these values are opportunity for all, economic growth, and the security of our families. HHS has a very important role to play in each of these areas.

Opportunity for all begins at home. Every child deserves the opportunity of a healthy start. And as the President reminded us in his State of the Union, research shows that one of the best investments we can make in a child's life is high-quality early education.

Studies show that the return on early education investments is at least 7 to 1, far exceeding any investment in the stock market.

Our budget puts a special focus on a birth-to-kindergarten pathway. It expands Early Head Start Child Care Partnerships so we

can give more children access to high-quality preschool and child care. And if you move forward with the President's Opportunity, Growth, and Security Initiative, with an additional investment which could be paid for by closing tax loopholes, we provide an additional 100,000 children with access to high-quality early education.

The budget before you also invests and empowers children's first and best teachers, their parents. It does so by expanding voluntary home-visitation initiatives, which are fully paid for by a tobacco tax. Not only are we able to help more children and their parents without adding a dime to the deficit, but we will be able to discourage more of our children from smoking.

Now, we know that the tobacco tax deters would-be smokers, particularly young smokers. We also know that 3,000 young Americans a day try their first cigarette and 1,000 of them become daily smokers. Therefore, we are also investing in more prevention, education, and media campaigns that have been shown to deliver results. We believe that we can make this generation a tobacco-free generation if we are willing to take action.

Early childhood and tobacco prevention efforts are important strategies for expanding opportunity and providing families with security. Affordable health care is another. No one can start a new business or save for retirement when they are drowning in medical bills.

This budget protects the progress we have made in the last 4 years to expand the opportunity of more affordable health coverage to more Americans. Through the end of February, 4.2 million Americans signed up for affordable health insurance plans through the Marketplace. And, as you know, these are private plans in a private market. We expect this number to rise by the March 31st deadline as more Americans learn just how affordable health coverage really is.

This budget is a job-creator. It bolsters some of the most important sectors of our Nation's innovation economy by investing in the NIH-funded BRAIN initiative, vaccine development, and other cost-cutting projects. It also creates jobs by increasing our primary-care workforce through investments in the Healthcare Workforce Initiative and the National Health Service Corps.

Ultimately, we all agree that there can be no opportunity without security, and the investments we are requesting in ASPR, CDC, and NIH matter to the security of every family. This budget requests funds to advance the development of medical countermeasures against chemical, biological, and radiological threats. We also move influenza preparedness forward, as well as vaccine development and the search for antivirals that are effective against drug resistance and virus mutation.

In addition, because no American should get sick as a result of a hospital stay, the budget invests in CDC and AHRQ's work to protect hospital patients from healthcare-associated infections.

This budget also protects the security of some of our most vulnerable populations. We expand elder justice initiatives that protect our parents from abuse, neglect, and exploitation. We support the Ryan White HIV/AIDS Program so we can expand access to care

and condition management to half a million lower-income Americans living with HIV and AIDS.

We make these investments while also making tough, fiscally responsible choices. Our budget contributes \$369 billion to deficit reduction over the next 10 years.

And we will, as Representative DeLauro has said, continue to fight waste, fraud, and abuse. Every dollar we have invested in the Health Care Fraud and Abuse Control Initiative, has recovered \$8.10. Last year, that totalled a record-breaking \$4.3 billion.

In summary, this budget expands the opportunity to more Americans, including the opportunity of a healthy childhood, the opportunity of affordable health coverage, and the opportunity of a job.

And, with that, Mr. Chairman, I would be pleased to answer the committee's questions.

Mr. KINGSTON. Thank you, Madam Secretary.

[The information follows:]



**STATEMENT OF
KATHLEEN SEBELIUS
SECRETARY
U.S. DEPARTMENT OF HEALTH AND HUMAN SERVICES**

ON

THE PRESIDENT'S FISCAL YEAR 2015 BUDGET

**BEFORE THE
COMMITTEE ON APPROPRIATIONS
SUBCOMMITTEE ON LABOR, HEALTH AND HUMAN SERVICES,
EDUCATION, AND RELATED AGENCIES
UNITED STATES HOUSE OF REPRESENTATIVES**

MARCH 13, 2014

Chairman Kingston, Ranking Member DeLauro, and Members of the Committee, thank you for the invitation to discuss the President's FY 2015 Budget for the Department of Health and Human Services (HHS).

This budget for HHS improves the economic opportunity of all Americans by providing critical investments in scientific research, health care, disease prevention, social services, and children's well-being, to achieve healthier families, stronger communities, and a thriving America. While it invests in areas that are critical to our long-term prosperity, the budget also helps tackle our deficit with legislative proposals that would save an estimated net \$356 billion over 10 years. For the activities funded by this Subcommittee, the Budget proposes \$69.7 billion in discretionary budget authority, a reduction of \$1.5 billion from FY 2014 enacted. With this funding, HHS will continue to improve health care and expand coverage, create opportunity and give kids the chance to succeed, protect vulnerable populations, promote science and innovation, protect the nation's public health and national security, and focus on responsible stewardship of taxpayer dollars.

Strengthening Health Care and Continuing Effective Implementation of the Affordable Care Act

Expanding Health Insurance Coverage. As of January 1, 2014, millions of Americans gained access to new health insurance options previously not available to them. The Marketplaces provide improved access to insurance coverage, creating a new private health insurance market in which those in need of coverage are more easily able to purchase health insurance. Minority communities will have increased opportunity for affordable health insurance coverage. Under the Marketplaces, 10.2 million uninsured Latinos, 6.8 million uninsured African Americans and over half a million uninsured American Indians and Alaska Natives will have new opportunities for coverage. As of March 1, 2014, the Marketplaces had enrolled more than 4.2 million individuals. New premium tax credits and rules ensuring fair premium rates are making private coverage more affordable for consumers. The Budget supports continued operations in the federally facilitated Marketplace, as well as oversight and assistance to state based and Partnership Marketplaces.

The Affordable Care Act provides full federal funding to cover newly eligible adults in states that expand Medicaid up to 133 percent of the federal poverty level for three years starting in 2014 and covers no less than 90 percent thereafter. The Affordable Care Act also simplified Medicaid and Children's Health Insurance Program (CHIP) eligibility and enrollment processes and aligned them with Marketplaces. The Centers for Medicare & Medicaid Services (CMS) continues to work with

states and other partners to advance state efforts that promote health, improve the quality of care, and lower health care costs.

Also beginning in 2014, consumers will benefit from a number of new protections in the private health insurance market. Non-grandfathered health plans will no longer be allowed to charge more or deny coverage to people because of pre-existing conditions. These new protections will also prohibit non-grandfathered plans from putting annual dollar limits on benefits and from varying premiums based on gender or any factor other than age, tobacco use, family size, or geography. In addition, new plans in the individual and small group market will be required to cover a comprehensive package of items and services known as Essential Health Benefits, which must include items and services within ten benefit categories. Finally, many individuals will find it easier to participate in clinical trials because issuers will have to cover their routine patient costs and cannot deny their participation in trials. This protection applies to all clinical trials that treat cancer or other life threatening diseases.

Health Centers. Health centers will continue to be a vital source of primary care for uninsured and medically underserved patients seeking a quality source of care in FY 2015. The Budget requests \$4.6 billion for health centers, \$3.6 billion of which is funded by the Affordable Care Act's Community Health Center Fund, to serve approximately 31 million patients in FY 2015. These resources will support the establishment of 150 new health center sites as well as enhance quality, and support capital development and facility improvements at currently existing health centers.

Protecting Vulnerable Populations

Elder Justice. The FY 2015 Budget proposes \$25 million in the Administration for Community Living (ACL) to protect vulnerable older adults by combating the rising scourge of elder abuse, neglect, and exploitation in America. This effort builds on the findings and recommendations of the Elder Justice Coordinating Council, a consortium of federal partners which I lead that was established by the Elder Justice Act of 2009. In response to the recommendations of the Council, ACL will begin developing a national Adult Protective Services data system and provide funding for key research. This investment will help states improve the quality and consistency of their Adult Protective Services programs.

Ryan White HIV/AIDS Program. Serving over half a million low-income people with HIV/AIDS annually, the Ryan White HIV/AIDS Program plays a critical role in supporting patients across the HIV continuum and ensuring care across all lifecycles, genders, and ages. The Budget

requests \$2.3 billion in FY 2015 to continue linking patients to care, engaging and retaining patients, prescribing and improving adherence to antiretroviral medicine, and achieving viral suppression.

Advancing Scientific Knowledge and Innovation

Promoting Global Health Security. Epidemic threats to national security arise at unpredictable intervals and from unexpected sources. The FY 2015 Budget includes an increase of \$45 million for global health security activities in the Centers for Disease Control and Prevention (CDC) to strengthen the capacity to prevent the introduction and spread of global health threats. CDC will help other nations build capacity to manage emerging threats, enhance early disease detection, improve disease confirmation, and effectively respond to epidemics and other public health catastrophes before they reach our borders.

Combating Antibiotic Resistance. While antibiotic resistance is not a new phenomenon, the current magnitude of the problem and the speed in which new resistance is developing pose the possibility of a future without effective treatment options. The Budget includes an increase of \$30 million for CDC's Detect and Protect Against Antibiotic Resistance initiative, which will enhance surveillance and laboratory capacity at local, state, and national levels to characterize domestic threats and protect patients from imminent danger.

In addition, the Biomedical Advanced Research and Development Authority (BARDA) anticipates spending \$79 million on its Broad Spectrum Antimicrobials program in FY 2015. Throughout the next several years, BARDA plans to build a portfolio in this area of candidate countermeasures, focus on developing applicable drugs, and obtain regulatory approval for use within hospital and community based settings.

Protect Patients from Healthcare-Associated Infections. The CDC estimates that one in 20 hospitalized patients acquires a healthcare-associated infection (HAI), and over one million HAIs occur across the healthcare spectrum each year at a cost of over \$30 billion. HHS is committed to reducing the national rate of HAIs. The Budget includes \$44 million for HAI prevention activities at CDC, which include identifying emerging threats and protecting patients through outbreak detection and control, laboratory testing of the health care environment and contaminated products, and guideline development.

Complementing CDC's efforts, the Agency for Healthcare Research and Quality (AHRQ) focuses on conducting research to develop new methods of preventing and reducing HAIs, and

disseminates these research findings to clinicians. The request includes \$34 million for AHRQ's efforts to protect patients from HAIs.

Advancing Biomedical Research. The FY 2015 Budget includes \$30.4 billion for the National Institutes of Health (NIH), an increase of \$211 million over FY 2014, reflecting the Administration's priority to invest in innovative biomedical and behavioral research that advances medical science while stimulating economic growth. In FY 2015, NIH will focus on generating the basic science for tomorrow's health breakthroughs, translating these basic discoveries into tailored and more effective health interventions, and nurturing diverse scientific talent and creativity.

BRAIN Initiative. In FY 2015, NIH plans to spend \$100 million on research collaborations with academic institutions, the private sector, and other government agencies on the Brain Research through Application of Innovative Neurotechnologies (BRAIN) initiative. This project will develop new tools to comprehensively and precisely examine the activity of the millions of nerve cells, networks, and pathways in the brain in real time to gain revolutionary understanding of complex brain functions and their links to behavior and disease.

Big Data. NIH will continue to ramp up efforts in FY 2015 to improve its ability to analyze many of the large and complex digital datasets of information, known as "Big Data," that biomedical researchers are currently generating, such as high-resolution medical images, recorded physiological signals, and complete DNA sequences of large numbers of individuals. Improving the ability to use, protect, and responsibly share such data, including the development of a well trained workforce, represents a critical link in translating new research discoveries into clinical applications.

Improving Healthcare through Meaningful Use of Health IT. Health information technology is essential to improving our nation's health care by moving from a transaction based system to one that emphasizes quality and value. The Budget includes \$75 million for the Office of the National Coordinator for Health IT (ONC) to coordinate and support investments in policies, standards, testing tools, and implementation guides that have dramatically accelerated the adoption and meaningful use of certified Electronic Health Record technologies. Within this total, ONC will begin to address HIT-related patient safety issues under the Health IT Safety Center through data collection and analysis on the types and frequencies of health IT related adverse events. ONC will work closely with AHRQ, Patient Safety Organizations, the Joint Commission, and FDA on this effort.

Supporting Families

Early Head Start—Child Care Partnerships. The Budget proposes \$650 million in FY 2015 for Early Head Start – Child Care Partnerships, an increase of \$150 million above FY 2014. These funds will provide access to high-quality early learning programs for tens of thousands of infants and toddlers through competitive grants to new and existing Early Head Start programs that partner with child care providers, especially those receiving federal child care subsidies.

Unaccompanied Alien Children (UAC). By law, the Administration for Children and Families (ACF) must assume custody of all unaccompanied alien children apprehended by law enforcement who file claims to remain in the United States. ACF provides support to state licensed group homes to care for these children until ACF can place the children with sponsors. Since FY 2011, the annual number of arriving UAC has increased from 6,560 to an estimated 60,000, for FY 2014. ACF has implemented strategies to reduce the cost per child, but total costs have risen dramatically as the number of UAC has increased. Due to the volatile nature of this program, the Administration is not able to reliably predict the number of UAC who will arrive in FY 2015 at this time. The FY 2015 Budget for the UAC program is therefore \$868 million, the same as FY 2014.

Facilitating Transitions to Adulthood

Youth Transitions. The FY 2015 Budget proposes to better serve the most vulnerable youth. When youth are disconnected from school, work or family they cost the nation billions of dollars every year in lost earnings, welfare and medical costs, and unmet personal potential.

The Budget includes \$130 million within the Substance Abuse and Mental Health Services Administration (SAMHSA) for the President’s Now is the Time initiative. This investment provides \$20 million to continue the Healthy Transitions program, which will assist 16 to 25 year olds with mental illnesses and their families in accessing and navigating behavioral health treatment systems to ensure their vulnerability does not hinder their treatment. The Budget also provides \$5 million within the Administration for Community Living to develop best practices and an evidence base to better support young people with intellectual and developmental disabilities as they transition from adolescence into young adulthood across all systems—health, education, employment, human services, and community living.

Protecting the Public's Health

Project BioShield and Advanced Development. In FY 2015, HHS will continue to support the development and procurement of medical countermeasures against chemical, biological, radiological, and nuclear threats. The Budget includes \$415 million to support advanced research and development through the Biomedical Advanced Research and Development Authority and \$415 million to develop and procure new measures through Project BioShield. Together, these efforts will improve the nation's ability to prepare for and respond to the most pressing threats.

Pandemic Influenza. The 2013 outbreak of the novel avian influenza virus, H7N9, demonstrated the critical need for sustaining and enhancing the nation's influenza preparedness and response capabilities. The Budget includes a total investment of \$170 million in the Public Health and Social Services Emergency Fund to support pandemic flu activities. Of this amount, \$73 million supports the advanced development of a universal influenza vaccine designed to be effective against all strains of flu to protect Americans from an influenza pandemic. The Budget also supports the advanced development of a new class of antivirals to improve effectiveness against virus mutation and drug resistance, and activities to improve vaccine manufacturing and production efficiency. These efforts, together with the pandemic influenza activities in CDC, NIH, and FDA, will improve the nation's protection against future novel influenza strain outbreaks.

Supporting Prevention

Reducing Tobacco Use. Having set a priority goal to reduce annual combustible tobacco use in the United States, HHS continues to make progress in reducing tobacco consumption and encouraging cessation among current users. The FY 2015 Budget includes \$211 million for CDC to implement comprehensive tobacco control and prevention activities, enhance educational efforts, expand the Tips from Former Smokers national mass media campaign, and increase tobacco cessation quitline capacity. HHS will continue work through the Tobacco Control Implementation Committee to align Departmental strategies with the Healthy People 2020 objective and the HHS Tobacco Control Strategic Plan in order to most effectively lead the nation toward a tobacco free generation.

Preventing Prescription Drug Overdose. The Budget includes \$26 million for new interventions to fight prescription drug misuse, abuse, and overdose. This investment includes a \$16 million increase for CDC to expand the existing State Core Violence and Injury Prevention Program to additional states with a high burden of prescription drug overdose to enhance their infrastructure and implement a structured set of interventions. These interventions will help to understand the nature of

the epidemic unique in each state, leverage the best available evidence to save lives, and adopt foundational overdose prevention practices. Also within the total, the Budget includes a \$10 million increase for SAMHSA to help state substance abuse authorities develop comprehensive prevention approaches through collaboration with state partners and integration of health information exchange systems with strategic plans.

Continuing Program Integrity and Oversight

Combating Fraud, Waste, and Abuse in Health Care. Last month, the Attorney General and I released the annual Health Care Fraud and Abuse Control (HCFAC) report showing that for every dollar spent on health care-related fraud and abuse investigations through this and other programs in the last three years, the government recovered \$8.10. This is the highest three-year average return on investment in the 17-year history of the HCFAC Program. The FY 2015 Budget continues to make cutting fraud, waste, and abuse a top Administration priority. In addition to the base discretionary (HCFAC funding in FY 2015, the Budget seeks new mandatory funding. Starting in FY 2016, the Budget proposes that all new HCFAC investments be mandatory, consistent with levels in the Budget Control Act.

To help ensure the prudent use of federal funds, the Budget also includes \$25 million in discretionary HCFAC funding for program integrity activities in private insurance, including the Health Insurance Marketplaces.

The Budget includes \$400 million in discretionary and mandatory funding for the Office of Inspector General (OIG), an increase of \$105 million above FY 2014. This increase will enable OIG to expand CMS Program Integrity efforts for the Health Care Fraud Prevention and Enforcement Action Team and improper payments, and also enhance investigative efforts focused on civil fraud, oversight of grants and the operation of Affordable Care Act programs.

The Budget also includes \$100 million for the Office of Medicare Hearings and Appeals (OMHA), an increase of \$18 million above FY 2014. The Budget will support adjudicatory capacity and central operations case processing in order to address a critical backlog in the number of appeals and maintain the quality and accuracy of its decisions.

Responsible Stewardship of Taxpayer Dollars

Contributing to Deficit Reductions while Maintaining Promises to all Americans. The FY 2015 Medicare and Medicaid legislative proposals seek to strengthen these programs through payment

innovations and other reforms that encourage high quality and efficient care while continuing to reduce health care cost growth. Medicare savings would total \$407 billion over 10 years by encouraging beneficiaries to seek value in their health care choices, strengthening provider payment incentives to promote high-value, efficient care, and lowering drug costs. The Budget includes \$7.3 billion in savings over 10 years to make Medicaid more flexible, efficient, and accountable. Together, the FY 2015 legislative proposals allow HHS to support the Administration's complementary goals of investing in the future and establishing a sustainable fiscal outlook.

Opportunity, Growth, and Security Initiative

The Budget proposes a \$56 billion, government-wide initiative to support both domestic and security expenditures that reflect the President's priorities to grow the economy and create opportunities. Resources for the initiative would be offset with a balanced package of spending reductions and the closing of tax loopholes. Multiple, specific HHS programs would benefit from the initiative.

National Institutes of Health. An additional \$970 million would be provided by the initiative to increase the NIH budget to \$31.3 billion. Funds would be used to increase the number of new grants funded by 650, and provide additional resources for signature activities such as the BRAIN Initiative, improving the sharing and analysis of complex biomedical data sets, expanding research on Alzheimer's disease and vaccine development, further accelerating partnership efforts to identify and develop new therapeutic drug targets, and other innovative projects.

Head Start. The initiative would also provide an additional \$800 million to further expand Early Head Start – Child Care Partnerships. This investment would bring total funding for Early Head Start – Child Care Partnerships to \$1.5 billion in FY 2015, and provide access to high-quality early learning programs for a total of more than 100,000 children.

Universal Influenza Vaccine Development. The initiative would provide an additional \$50 million to support the advanced development of vaccine candidates for a universal influenza vaccine and to support activities to improve the basic effectiveness of existing vaccines. Within the Public Health and Social Services Emergency Fund, this investment would bring total funding for universal influenza vaccine development to \$123 million in FY 2015.

Thank you for the opportunity to testify. I will be happy to answer any questions you may have.

AFFORDABLE CARE ACT AND CYBERSECURITY

Mr. KINGSTON. I was watching an interview with somebody who was knowledgeable of cybersecurity, and he said that the Obamacare Web page or the exchange sites—and I know there are different ones, but he said it was 4 minutes away from being able to be hacked by the average hacker to get income information, healthcare information, family information.

Do you agree or disagree with that? And how secure are the Web pages?

Secretary SEBELIUS. Well, cybersecurity is certainly a huge issue and priority, and it is a huge issue for us.

And, Chairman Kingston, first of all, we collect no health information because it is currently not needed because insurance companies can no longer lock anybody out with health information. So that is not collected.

Secondly, the Hub, which is the central focus of both State-based Marketplaces and the Federal Marketplace, doesn't store any information. It is a router to ping other secure government systems and deliver back information.

But, finally, the website, the Federal Marketplace and the States, have been built to the highest Federal standards. We have ongoing and continuous penetration testing. As recently as mid-December, we conducted a full security control analysis and had a green light to go—no concerns were found in the end-to-end testing that was performed in the secure lockdown site. We have continuous testing not only from HHS outside entities but, on an ongoing basis, penetration testing. And there has been no successful malicious attempt to get personally identifiable information.

But we are continuing to improve the site. I think the private-sector site breaches that we heard about late in the year with a number of top retailers have sent shock waves through everyone. And I think it is incumbent on all of us, not just with the new Marketplace, but we also run the Medicare system; we have Medicaid information; so we have a lot of personally identifiable information. It has always been a priority, but, believe me, we are working on a continuous basis to increase our security efforts.

AFFORDABLE CARE ACT IMPLEMENTATION

Mr. KINGSTON. Okay.

A question about the implementation. I know that there was part of the law when it was passed that said that the White House could waive certain provisions in order to implement it. But now that the law has been the law of the land for 3 years—

Secretary SEBELIUS. Four almost.

Mr. KINGSTON [continuing]. Four, I don't understand the authority in which the administration uses to waive certain requirements on mandates. And how many mandates have been waived? I hear 20, I hear 27, I hear 28.

Secretary SEBELIUS. Well, Mr. Chairman, first of all, I think this has been a multiyear, as we just discussed, implementation effort. And what we are attempting to do is have a smooth transition into the new Marketplace. Our agency as well as other departments across the Federal Government have fairly broad discretionary au-

thority in terms of implementation efforts. And, at each point along the way, we have gotten legal counsel approval for the steps we have taken.

Nothing has been discarded, in terms of the law. The law is still very much in place. What we are doing with some of the features of the law, is having a transition most focused on people who have insurance coming into compliance with some of the new features of the plans, and to gradually phase those in over a period of time. But they will all be in place, they will all be enforced, and they are all still very much a part of the law.

Mr. KINGSTON. Could you provide us with that legal opinion?

Secretary SEBELIUS. I would be happy to.

[The information follows:]

Chairman Kingston: Could you provide us with that legal opinion? Full context of question begins on transcript pages 17-18 (lines 358-386).

While there is not a specific opinion from our General Counsel, below is the explanation of our authority that has supported these actions.

The Supreme Court held more than 25 years ago that agencies charged with administering statutes have inherent authority to exercise discretion to ensure that their statutes are enforced in a manner that achieves statutory goals and are consistent with other administrative policies. Agencies may exercise this discretion in appropriate circumstances, including when implementing new or different regulatory regimes, and to ensure that transitional periods do not result in undue hardship.

The case is *Heckler v. Chaney*, 470 U.S. 821 (1985).

Mr. KINGSTON. And could you do it today?

I just don't want it to disappear.

Secretary SEBELIUS. Sure. It won't disappear, but I will do it as quickly as we can.

Mr. KINGSTON. Okay.

I am going to yield to you, Rosa, because of the time.

Thanks.

AFFORDABLE CARE ACT

Ms. DELAURO. Thank you, Mr. Chairman.

Madam Secretary, I have spent my entire career fighting to ensure universal access to high-quality, affordable health care. And I want to again say thank you to you for your hard work to bring this Nation closer to that reality.

I have three or four questions, of which I would like to have you just confirm. There is, at this juncture, no need for lengthy answers here.

Question: For instance, isn't it true that about 3 million young adults have coverage today because the ACA allows them to stay on their parents' plans?

Secretary SEBELIUS. There are about 7 million who are staying on their parents' plan. The insurers tell us at least 3 million of those folks had no insurance prior to being on their parents' plan.

Ms. DELAURO. Thank you.

Isn't it true that the American consumers have saved \$1.5 billion in premium costs due to the Affordable Care Act?

Example: Medical loss ratio requires insurance companies to spend at least 80 percent of their collections from premiums on providing actual healthcare services as opposed to administrative costs, marketing, or salaries for high-paid executives. Hasn't this resulted in significant rebates to American families?

Secretary SEBELIUS. Congresswoman, consumers have gotten rebates, small-business owners have gotten rebates, as well as, using the new authority that they have, a number of State insurance commissioners have turned down what were double-digit rate hikes and made it very clear that they are not allowing those. So both have happened.

Ms. DELAURO. That last piece was true of the State of Connecticut, in a double-digit rate hike.

Question: Isn't it true that seniors are saving almost \$1,000 per person on drug costs due to the Affordable Care Act, due to closing the donut hole in Medicare Part D?

Secretary SEBELIUS. Well, the seniors who qualify for the donut hole because they purchase those prescriptions have saved at least \$1,000 a piece, on average, because of the ACA provisions, yes, ma'am.

Ms. DELAURO. One of the most important pieces of the Affordable Care Act is its focus on increasing access to preventive services. Isn't it true that more than 25 million Medicare beneficiaries are receiving free preventive care as a result of the Affordable Care Act?

Secretary SEBELIUS. Well, as you know, Congresswoman, not only do seniors receive that, but now insurance policies offer preventive services with no copays and no coinsurance. So cancer

screenings and flu shots and children's immunizations are all part of insurance benefits.

Ms. DELAURO. Thank you.

Last 3 years, real per capita annual growth of national health expenditures is only 1.3 percent, less than a third of the long-term historical average, lower than the previous 3-year period, which coincided with the recession. There are many moving parts and pieces in the U.S. economy, but I think we could say that the Affordable Care Act has been successful in constraining the growth of healthcare spending.

Can you talk about the impact the ACA is having on healthcare costs and healthcare spending? If the ACA continues to constrain the growth of healthcare expenditures, won't that wipe out a large portion of the projected future deficit?

Secretary SEBELIUS. Well, Congresswoman, we are seeing the lowest healthcare increases, some people say, in recorded history.

Medicare is growing at a slower rate. It was up over 6 percent year-in and year-out in the decade before the ACA was passed. In 2010 to 2012, it grew at a rate of 1.6 percent per capita. Last year, 0.7 percent per capita—a rate never seen in the 50 year history of the program.

Medicaid costs across the country are rising at about half the rate that they did prior to the ACA. If you compare the decade before and the 4 years since, it is about half.

Overall health expenditures for the United States are rising at half the rate they did in the decade before as compared to the 4 years since the ACA. And private insurance rates are rising at about half the rate.

Ms. DELAURO. Uh-huh. So, overall, we are seeing a bending of that healthcare cost curve.

Secretary SEBELIUS. Significant. And, initially, people said this was related to the recession. Health economists now are saying there is some fundamental transformation going on in the overall healthcare expenditures. And that is very good news.

Ms. DELAURO. Okay. Thank you.

HEALTH CARE FRAUD AND ABUSE

I don't know if—well, let me move to the healthcare fraud and abuse, which you addressed in your commentary. I mentioned this in my opening statement. The Budget Control Act of 2011 authorizes two cap adjustments in program integrity initiatives in the Labor-HHS bill. The cap adjustments are provided for programs that actually reduce the budget deficit by preventing fraudulent expenditures in Federal programs. Health Care Fraud and Abuse Control Program is one of these programs, estimated to save nearly \$8 in taxpayer money for every dollar spent.

Can you tell us about the fraud-prevention activities that HHS could be pursuing this year but can't because of the fiscal year 2014 bill that didn't fully fund this program?

Let me just leave it there, because my time might be running out.

Secretary SEBELIUS. Well, part of the lesser-known features of the Affordable Care Act is it is probably one of the toughest anti-fraud measures ever passed by the United States Congress. You

gave us a lot of new tools. You increased criminal penalties for fraudulent activity, gave us new resources to set up predictive modeling so we can do what the private sector does, which is look at expenditures. And additional resources were used to expand the very successful on-the-ground strike forces of the Justice Department working with our fraud investigators. That, combined, has increased the number of arrests and trials and recoveries, so last year we announced \$4.3 billion was put back in both the Medicare and Medicaid Trust Funds thanks to those efforts.

So we would be able to expand strike forces, do more vigilant activity. Medicare is a huge program, as is Medicaid. Fraud activity occurs. And the further we can get out ahead of it, and not pay and chase, the better off we are going to be.

Ms. DELAURO. Can you just deal with what kind of money we are talking about, what kind of savings? Is that possible to predict?

Secretary SEBELIUS. Well, we have now over the last couple of years returned 8 to 1 so for every dollar spent, \$8 is put back in the Trust Fund.

Mr. KINGSTON. The gentlewoman's time has expired.

Ms. DELAURO. Thank you. Eight to 1. Thank you.

Mr. KINGSTON. Mr. Fleischmann?

Mr. FLEISCHMANN. Mr. Chairman, I understand that you wanted me to yield about a minute of my time, sir?

Mr. KINGSTON. Let me just say this for the record to my friend, Ms. DeLauro, and for the Secretary: Your budget last year was \$5.3 billion, and this year it is 23. That is not a decrease. Going from \$5.3 billion to \$23 billion is not a decrease in healthcare costs. Now, we can quote all this stuff and have all these nice rhetorical exchanges, but the numbers don't show that at all.

Mr. Fleischmann, thank you.

Mr. FLEISCHMANN. Thank you, Mr. Chairman.

AFFORDABLE CARE ACT ENROLLMENT

Good morning, Madam Secretary.

Your administration has repeatedly given reprieve to business by delaying the mandate that requires large employers to provide healthcare coverage or pay fines, yet you refuse to consider granting that same option to individuals who are struggling to meet the requirements of Obamacare.

In fact, your agency stated on Tuesday that you do not have—and I repeat, you do not have—the statutory authority to delay the enrollment deadline. And in your testimony before the House Committee on Ways and Means yesterday, you stated that the administration will not delay the individual mandate or extend the 6-month open enrollment period scheduled to end March the 31st.

Madam Secretary, I would like you to clarify whether you can think of any reason—and I state, any reason—HHS would delay the March 31st deadline for enrollment, SHOP exchanges, or any other Obamacare deadlines. Please provide this subcommittee and the American people with a straight answer.

And I want a yes-or-no answer: Will you or will you not delay the individual enrollment deadline on any other aspect of Obamacare?

Secretary SEBELIUS. The enrollment deadline will not be delayed, as I said yesterday.

The SHOP doesn't have a deadline. Small-business owners can sign up at any point, so they don't operate in an open enrollment period. That is the way that insurance market works. They don't have to worry. Anybody eligible for Medicaid can sign up at any time, as can small-business owners.

But the enrollment deadline, which was set out to end March 31st, will end March 31st.

Mr. FLEISCHMANN. Okay. So then we agree that there is no statutory authority to extend these deadlines and that they will not be extended.

Secretary SEBELIUS. The enrollment deadline will be March 31st.

Mr. FLEISCHMANN. Thank you.

Mr. Chairman, I yield back.

Mr. KINGSTON. Ms. Roybal-Allard.

Ms. ROYBAL-ALLARD. Welcome, Madam Secretary.

MINORITY HEALTH

As you know, minority health in our country suffers disproportionately from the rest of the population. So it is important that every effort is made to reduce the racial and ethnic health disparities that exist in our country.

Yet, in your proposed budget, CDC's Racial and Ethnic Approaches to Community Health, the REACH Initiative, which funds community-based programs and culturally tailored interventions to address health disparities, is eliminated. Programs that focus on healthcare workforce diversity, such as the Health Careers Opportunity Program and area health education centers, are also eliminated. And on top of the elimination of these programs, the budget of the Office of Minority Health is scheduled to be cut by 37 percent.

I understand that these cuts are to replace REACH grants with grants that focus on chronic disease. How will these new grants replace the work and accomplishments of the REACH program? And what is the rationale for eliminating proven workforce diversity programs like HCOP and AHEC and for cutting the Office of Minority Health budget by 37 percent, which is about \$21 million?

Secretary SEBELIUS. Well, Congresswoman, we share your focus and attention on reducing health disparities and providing funding for programs and services to improve health in minority communities. And let's just start with the Affordable Care Act, which has had a significant impact, and probably the most significant impact on reducing disparities, since African Americans and Hispanics are more uninsured, by population, than their white neighbors and friends.

But the budget has \$11.9 billion for programs and services to improve the health in minority communities. We have an additional \$960 million going out to community health centers with new access points and can serve up to 31 million patients. Sixty-two percent of the health center patients are racial and ethnic minorities.

There is new money going into the Indian Health Service, one of the least-served populations, to continue projects and reduce health

disparities. And additional money will be invested into the Ryan White HIV/AIDS program.

CDC suggests that, rather than funding the REACH program, that new partnerships in community health, and grant programs, as well as the chronic disease and prevention programs' funding announcements will more than cover not only that target population, but they think it will do it more efficiently than REACH.

And while there is a decrease in the Office of Minority Health, I would say that the grants are coming to a natural end in that office, and we are looking at broader service programs that can pick up that focus and effort.

Finally, we do have increased funding, which I think is critically important, in the workforce areas to make sure that we further diversify our workforce. So in HRSA, in our effort to more than double the size of the National Health Service Corps, where currently over 30 percent of the new Corps members are minorities. We think that that will continue. More than half of the 1,100 Corps members in the pipeline are minorities. And that will make sure that people are actually in a more diversified workforce setting than we have ever had before in the history of this country.

Ms. ROYBAL-ALLARD. I think that the concern is that some of the programs that you are talking about deal more with current health providers and, you know, enhancing their work. And the concern at least that I have is that the programs that you are cutting are mostly about recruiting minority health providers and building a pipeline for future healthcare workers.

So I guess my question would be, then how do these programs that you have just mentioned specifically address recruitment and retention of minority health providers, and how do they build this pipeline that is going to be so critical in the future?

Secretary SEBELIUS. Well, again, I would say that one of the most successful programs that we have is the National Health Service Corps. Thirty percent of the National Health Service Corps members who receive scholarship and loan repayments are minorities.

We currently have about 8,900 National Health Service Corps members in the country. This budget would bring that number to 15,000 and keep it at 15,000. That is a whole lot of new pipeline. And we will very much double down on the effort to make sure that—

Mr. KINGSTON. Mr. Joyce.

Secretary SEBELIUS [continuing]. Minorities are overrepresented in that population. That is a new group of healthcare providers.

Mr. KINGSTON. Mr. Joyce.

Mr. JOYCE. Thank you.

SMALL BUSINESS HEALTH OPTIONS PROGRAM

Madam Secretary, CMS rules require the Federal exchange and all State exchanges to implement a Small-Business Health Options Program, otherwise known as SHOP, that provides an employer the ability to make available to their employees all exchange health plans at a meta level—for example, bronze, silver, gold, or platinum.

This employee-choice model is administratively complex. In 2013 and 2014, several States attempted to implement the employee-choice approach but encountered technical issues that required them to either take down or delay launching their SHOP program. Yet, CMS still requires all States to have this ready to go later this year.

An employee-choice SHOP is an enormous IT undertaking across multiple business partners and vendors to allow for online shopping, enrollment, automated employer billing, and payment of health plans. However, there is little transparency into CMS progress in implementing this new SHOP model for 2015. In particular, details on key milestones for development, testing, and availability have not been released outside the government. More transparency is critical, since CMS will be implementing SHOP in 37 States.

Based on media reports, enrollment in State SHOP exchanges is miniscule. However, CMS hasn't released any data on how successful it has been in enrolling employers to date.

Madam Secretary, how many employers are covered under the Federal SHOP today?

Secretary SEBELIUS. Sir, I don't have that number off the top of my head, but I can get it for you today.

[The information follows:]

Representative Joyce: How many employers are enrolled in the Federal SHOP? Full context of question begins on transcript pages 29-31 (lines 664-692). **Please note that during the exchange with Representative Joyce, the Secretary indicated she could get him the SHOP enrollment numbers. This information is not available. She later corrected herself on page 76 of the transcript. The information we do have available is directly below.

Although the metrics you requested are not currently available, we expect to have SHOP enrollment data at a later date and will provide you and your Committee that information when it is available. There are several challenges to providing meaningful data on enrollments in SHOP policies at this time, including the fact that employers may purchase SHOP policies at any point during the year instead of during a fixed open enrollment period, and employers are currently enrolling directly through issuers of group policies.

As you know, the SHOP Marketplace helps small businesses provide health insurance coverage to their employees. For 2014, the SHOP Marketplace is open to employers with 50 or fewer employees that offer coverage through the SHOP to all full-time employees, either in the SHOP where the employer's principal place of business is located, or in the SHOPS where their employees have their primary work sites. Generally, employers with fewer than 25 full time- equivalent employees (as determined according to guidelines established by the Department of the Treasury) may qualify for tax credits if they offer their employees insurance through a SHOP and meet other criteria established by the Department of the Treasury.

Small employers can enroll their employees in SHOP coverage throughout the year. As a result, SHOP enrollment is not limited to the individual Marketplace open enrollment period, and SHOP enrollment figures will vary throughout the year. The Centers for Medicare & Medicaid Services (CMS) is working to collect enrollment data from insurance companies.

It is also important to note that for federally-facilitated SHOP coverage beginning in 2014, small businesses do not need to apply for SHOP eligibility before enrolling in coverage, and they may enroll their employees in coverage through HealthCare.gov, or through an agent, broker or insurer that offers a certified SHOP plan and has agreed to conduct enrollment according to CMS standards for the Federally-facilitated SHOP Marketplace.

Mr. JOYCE. Well, thank you. I would appreciate that today.

Secretary SEBELIUS. Our last enrollment report would have it, and I just don't have the enrollment report with me.

Mr. JOYCE. I would appreciate that today.

Secretary SEBELIUS. Sure.

Mr. JOYCE. Thank you.

In light of the recent consumer experience rolling out the individual exchanges and the late announcement last November delaying SHOP on online enrollment for the Federal exchanges, what assurances can you provide small employers that the rollout of the FF-SHOP will be different from the individual consumer experience last October?

Secretary SEBELIUS. Well, Congressman, I have repeatedly said, and I will say it again, the launch in October was failed and flawed. The good news was that the consumer had a very different experience 8 weeks after October 1st, but that is no excuse for the 8 weeks of, really, failed technology.

We announced prior to the launch of the ACA that in the Federal Marketplace—States have made different choices with the State-based market—that while we would offer SHOP plans, we would not offer the second feature, as you say, employee choice. So an employer can choose among plans in the market in 2014, and a number are. And they are dealing with agents and brokers the way they have always dealt with small-business coverage this year.

We are on track to have an automated system which will allow us to go to step two in the Federal marketplace, so employees can actually choose between plans, which is a feature that a lot of small employers have never been able to offer. That technology will start to be built after open enrollment finishes.

And we can get you regular updates, but I just got a report from the technology team that they feel it is feasible to have it online by the time open enrollment starts on November 15th of 2014.

Mr. JOYCE. Well, see, Madam Secretary, last year when you were here, I asked you if there was a place that consumers or Americans could go to follow, somewhere within the government, the rollout of the Affordable Care Act. And I got a letter—

Secretary SEBELIUS. They followed it.

Mr. JOYCE. Yeah. Well, I got a letter back from you in August saying that, you know, you would get back to me. And, again, you know, we got to follow it, and it didn't follow out all that well in October.

Last year, CMS determined that it wouldn't be able to allow employees to select from any health plan on SHOP and would instead focus on allowing small employers to enroll in a single health plan. Then in November, CMS announced that it would not be capable of even processing an enrollment for employers. This meant that small employers that applied for coverage through the Federal SHOP had to start over and apply for coverage directly through participating health plans. That was very disruptive to small employers.

Shouldn't CMS prove that it can implement what it had planned for 2014 before attempting to implement the more complicated systems that allow employees to choose from among multiple health plans?

Secretary SEBELIUS. Well, that is exactly what we are doing. This year, we have a system in place—if a small employer wants to take advantage of the tax credit which is available to some small employers with low-wage workers, we have an arrangement with the insurers that they can qualify for the tax credit and enroll with insurers.

If a small employer wants to offer coverage the way they always have and has no interest in the tax credit or isn't eligible for the tax credit, they are enrolling as they choose this year. And, as I say, the automated version, including the employee choice, will be up and running.

Mr. JOYCE. Well, are CMS, its vendors, and business partners working under a coordinated Federal timeline? And if so, what are the deadlines and key milestones in that timeline?

Secretary SEBELIUS. For this plan, I, again, don't know that off the top of my head, but I can get that for you.

Mr. JOYCE. You can supply us those timelines?

Secretary SEBELIUS. Yes.

[The information follows:]

Representative Joyce: Well, are CMS, its vendors, and business partners working under a coordinated Federal timeline? And if so, what are the deadlines and key milestones in that timeline? Full context of question begins on transcript pages 33-35 (lines 732-774).

The Federally-facilitated SHOP is open to employers with 50 or fewer full-time-equivalent employees (FTEs) and enrollment is open year-round. In April 2014, CMS will announce the availability of two new tools on Healthcare.gov to make it easier for small employers to understand and take full advantage of the benefits available from the SHOP. A new FTE Calculator helps small employers determine whether they might be eligible to buy Federally-facilitated SHOP coverage for their employees. The FTE Calculator can be accessed at the following link: <https://www.healthcare.gov/fte-calculator/>. In addition, a new SHOP Tax Credit Estimator lets small employers learn the size of the tax credit they might, if otherwise eligible, be able to receive for contributing to coverage for their employees through the SHOP. The SHOP Tax Credit Estimator can be accessed at the following link: <https://www.healthcare.gov/small-business-tax-credit-calculator/>

Mr. KINGSTON. Ms. Lee.

Thank you.

Ms. LEE. Thank you, Mr. Chair.

Let me first say welcome and it is good to see you, Madam Secretary.

Secretary SEBELIUS. Thank you.

HEALTH DISPARITIES

Ms. LEE. Following up with Congresswoman Roybal-Allard's remarks—and I associate myself with all of her remarks with regard to the budget cuts as it relates to the cuts to the Office of Minority Health. One of the issues—Congresswoman Roybal-Allard, myself, and Congressman Honda helped negotiate the health disparities provisions in the Affordable Care Act for the specific purpose to really prioritize—of course, in a country that is rapidly becoming more diverse—to prioritize closing these gaps in communities of color.

And so I am really worried about these budget cuts and the fact that it doesn't really now appear that that is a priority, and, in fact, we are not going to make a lot of progress in this. So I hope that we go back to the drawing board and look at this very, very carefully.

Secretary SEBELIUS. Well, again, Congresswoman, I would suggest that the budget before you has an increase of about \$775 million in funding specifically for minority health issues, and that does not include any of the direct efforts through the Affordable Care Act to qualify people for health insurance.

So, in addition to that effort, there is an additional—and the programs include programs in HRSA, the Indian Health Service, and in the Office of Research and Quality. But we would be glad to enumerate those programs and get back to you.

Ms. LEE. It would be good to see—

Secretary SEBELIUS. Yes.

Ms. LEE [continuing]. Because, for instance, eliminating the funding for the REACH Program, eliminating funding for the centers for excellence, Healthcare Opportunities program, Area Health Education Centers—all of those have been reduced or eliminated. So we need to see how you are going to—

Secretary SEBELIUS. Absolutely. And in—

Ms. LEE [continuing]. Deal with this.

Secretary SEBELIUS [continuing]. Some of those cases, the leaders of those agencies have made a determination not to decrease their efforts in minority health but, actually, to expand them into broader programs that they thought would reach more of the population of interest.

Ms. LEE. Okay. Yeah, I hope we have a chance to walk through this.

Secretary SEBELIUS. And I would be glad to do that.

GUN VIOLENCE

Ms. LEE. Let me ask you about gun violence. You know, it has been more than a year now since the tragedy at Sandy Hook in Connecticut and a month-and-a-half removed from the deadly mall shooting in Maryland. Of course, Congress, for obvious reasons, has

failed to take a single step to address gun violence in America taking innocent lives every day. Despite overwhelming public support, we have really been unable to act on something as noncontroversial as background checks. And so, as we wait, according to the Children's Defense Fund, they estimate that more than 21,000 children and teens have been shot by guns since this Congress alone.

So I wanted to ask you about this study, the Institute of Medicine report that proposes a research agenda on gun violence, including key topics such as risk and protective factors and the characteristics of gun violence.

Are you doing any gun violence prevention research? How does this budget attempt to address this in your proposals? And how do you see this gun violence issue as a public health problem? Because many, many want CDC and others to begin to define at least part of it as a public health issue.

Secretary SEBELIUS. Well, I don't think there is much debate or dispute in the healthcare community that gun violence is a major public health problem and not only has a serious economic cost but a serious personal cost in the loss of lives.

In the President's "Now is the Time" agenda, some of those suggestions were dependent on congressional activity, some were Executive orders that could be carried out by departments. And for our department one of them was to ask both the NIH and the CDC to refocus on the issue. CDC will focus on surveillance of gun violence and produce data and information, which they are doing and will continue to do, and NIH will look at various causes and effects.

We also have efforts in Substance Abuse and Mental Health Services Administration that focus on not only the trauma that is created by survivors of gun violence and efforts to help victims, but a fairly significant increase in the 2014 budget and also for 2015 in the number of mental health professionals. I mean, a greatly underserved area is often early intervention in mental health issues that could identify problems at the front end.

Mr. KINGSTON. Dr. Harris.

Mr. HARRIS. Thank you very much.

PHS EVALUATION TAP

Let's turn our attention a little bit to the NIH budget, which I understand comes in at \$30.4 billion. And your statement says it increases \$211 million over fiscal year 2014, reflecting the administration's priority to invest in innovative biomedical and behavioral research.

But, in fact, in the same budget, you increase the tap from 2½ to 3 percent, taking back \$152 million of that \$211-million increase. Is that correct?

Secretary SEBELIUS. The tap has always been a part of our budget, yes, sir.

Mr. HARRIS. But you are increasing the tap—

Secretary SEBELIUS. We are increasing it, but that has always—

Mr. HARRIS [continuing]. Taking \$150 million more than you would under the old tap. Is that correct?

Secretary SEBELIUS. That is correct.

Mr. HARRIS. Okay. So basically the increase really, because of an accounting gimmick, is really only \$50 million.

The tap you take from NIH is valued at—you would propose a \$900-million tap? So 3 percent of \$30 billion? I mean, that is a back-of-an-envelope estimation.

Secretary SEBELIUS. I assume that is correct.

Mr. HARRIS. Well, I think the math of 3 percent of \$30 billion is \$900 million.

Secretary SEBELIUS. I just wasn't clear—

Mr. HARRIS. So if we—

Secretary SEBELIUS [continuing]. That \$30 billion was correct, sir.

Mr. HARRIS. If, in fact, your office didn't take the tap, you could triple Alzheimer research at the NIH, you could increase Parkinson's research eightfold by leaving that \$900 million in the NIH. I mean, you could double or increase 2½ times the breast cancer research. But instead you choose to take a 3-percent tap from the NIH.

And the interesting thing is you say your budget reflects the administration's priority to invest in innovative research, but, in fact, the best measure of the administration's priorities is, where does the tap money go? Because that really is your ability to set priorities of the administration.

Am I correct that in fiscal year 2013, the last number that I have available, zero tap dollars were sent back to NIH for medical research—zero?

Secretary SEBELIUS. I can't—

Mr. HARRIS. Out of—

Secretary SEBELIUS. I can tell you Congress appropriates virtually all of the tap from the HHS budget. So I think there are about \$40 million of discretionary funding. So if Congress did not direct us to send tap money to NIH, it probably isn't there.

Mr. HARRIS. Okay. So you would be okay if we directed tap money to the NIH—

Secretary SEBELIUS. As I say, Congress has—

Mr. HARRIS [continuing]. Because of the priorities?

Secretary SEBELIUS [continuing]. For years appropriated the money that comes in through the HHS—

Mr. HARRIS. So, I mean, you are not answering my question. So you would be okay if we directed that additional \$900 million that your budget takes out of the NIH budget, the tap, back to the NIH to restore medical research?

Secretary SEBELIUS. We will certainly work with Congress on whatever the direction is. But it is a congressional appropriation that—

Mr. HARRIS. Good.

Secretary SEBELIUS [continuing]. Has come in year-in and year-out to spend—

Mr. HARRIS. I understand, but—

Secretary SEBELIUS [continuing]. That money, so it is not our discretion.

Mr. HARRIS [continuing]. It was not our decision to increase the tap from 2½ to 3 percent. That was an administration decision in this budget.

Secretary SEBELIUS. I think that is correct, but——

Mr. HARRIS. I know that is correct——

Secretary SEBELIUS [continuing]. You do appropriate the money, sir.

MARYLAND HEALTH INSURANCE EXCHANGE

Mr. HARRIS [continuing]. Madam Secretary.

Listen, let's move on, because I only have a couple more minutes. The Maryland exchange is a disaster. Your Inspector General correctly agreed last week to inspect and to audit the Maryland exchange.

But my understanding is they are coming back to the Department for 30 million additional dollars to bail out an obviously failed exchange. Is it the intention of the Department to provide that additional emergency funding?

Secretary SEBELIUS. Sir, we do regular financial audits on all of the State-based markets. I am not aware of this request. We have all of the IT funding for IT that isn't working under specific restrictions. And we will look at their proposal and see where they are going forward.

Mr. HARRIS. When did the Department know that there were tremendous problems in the Maryland exchange?

The Department is about to invest a total of \$200 million, which, by the way, could double the Parkinson's research in the country if, instead of wasting it in the Maryland exchange, you had funded Parkinson's research.

When did the Department know about the failures in the Maryland exchange?

Secretary SEBELIUS. Sir, we got regular updates on when they launched——

Mr. HARRIS. So the Department knew? So the Department knew that this was a failure?

Secretary SEBELIUS. We did not know—we knew it was not working properly starting in——

Mr. HARRIS. Well, then, Madam Secretary, I hope you don't spend another \$30 million there.

EPIDIURALS

Finally, the last thing is, earlier this year, CMS implemented extremely steep cuts to payments for certain epidural pain procedures, procedures I am familiar with—I have never done them, but I am familiar with them—to treat low back pain. They decreased by 56 percent the payment for these. I will tell you, Madam Secretary, that the effect of decreasing that is to ration this procedure to seniors with back pain. That is the effect of cutting 56 percent of payments.

Due to a magnitude of the cuts, does CMS intend to revisit the decision through the agency's refinement process? You don't have to tell me now. If you can get back to staff within a couple of weeks, I would appreciate whether that is going to be revisited through the refinement process.

Secretary SEBELIUS. I could certainly ask that question.

Mr. HARRIS. Thank you very much, Madam Secretary.

I yield back.

Mr. KINGSTON. Mrs. Roby.

AFFORDABLE CARE ACT

Mrs. ROBY. Thank you for being here.

Over the last few months, thousands of health insurance policyholders in my home State of Alabama have received notice that their plans have been canceled or altered. The costs have risen, some quite dramatically. A family with one income's premium doubled from \$420 to \$940 a month.

That being said, there is already a 3.5 administrative fee imposed on residents of States where the exchange is run by the Federal Government, like Alabama. Mr. Cohen told Congress in previous testimony that the percentage might have to be increased next year.

Could you tell this committee when you expect to finalize the payment parameters rule for 2015?

Secretary SEBELIUS. Well, first of all, Congresswoman, there is no payment imposed on residents of your State. There is a fee on insurance companies—

Mrs. ROBY. That is passed on to the individuals.

Secretary SEBELIUS. If an insurance company chooses to do that, they can. If they choose to take it out of their profit margin, they can do that. They have rates already filed, so they can't pass it on. If rates are in the market, then they are locked in—

Mrs. ROBY. But we are seeing—as a result of this administrative fee, we are getting direct stories from these individuals of a dramatic increase. So, clearly, the fee plays a part of that.

Secretary SEBELIUS. And the rule has been finalized, so that is the fee that insurance companies will pay.

Mrs. ROBY. Do you believe it is reasonable to penalize these individuals with this administrative fee if their State was unable to operate, particularly because of insolvency, fiscal insolvency, especially these individuals that have already seen such a dramatic increase in their premiums?

Secretary SEBELIUS. Again, I am not sure who has fiscal insolvency. This is a fee on insurance companies, who are seeing millions of new customers come into their businesses because of the Affordable Care Act. And the fee is to get us to the point where the Marketplace will be self-sustaining. So the users and the companies, who will make profit based on their new customers, will actually be paying for the infrastructure to sell insurance products.

Mrs. ROBY. Right. And the reality, though, is that these dramatic increases are having an unbelievable effect on individuals that are having to make choices, quite frankly, between paying their mortgage or being able to pay for their health care.

And I am not making this up. They are calling my office. They are sending their premium statements, showing us. These are real lives that are being affected by these dramatic increases.

I want to ask you just a series of questions based on your testimony about the 4.2 million individuals that are enrolled already—below the 7 million individual goal. And I heard you say, I believe—correct me if I am wrong. You said—as far as enrollees on the exchange that were previously insured, did you state that number already?

Secretary SEBELIUS. I did not.

Mrs. ROBY. Okay. Do you know that number?

Secretary SEBELIUS. I do not.

Mrs. ROBY. Can you get back to us today, please?

Secretary SEBELIUS. Insurance companies have that number. I do not have that number.

Mrs. ROBY. Okay. How many enrollees have currently paid premiums?

Secretary SEBELIUS. I do not have that number. As I told the committee members yesterday, we have aggregate numbers that are a month old from insurers. We do not have individual data from insurers.

We will give you that as soon as we have it. I do not have accurate, timely information, and I can't give you that information. And we will never have, until the fully automated system for the financial payment is in place, numbers on the individuals who are customers, who are paying their premiums, who are not eligible for the APTC.

Customers pay the insurance companies. They do not pay the Federal Government. These are contracts between Blue Cross Blue Shield or Humana or Kaiser and——

Mrs. ROBY. So you don't know the cost per member or cost per month of the individual——

Secretary SEBELIUS. I can look on the website and tell you the cost per member and the cost per month. It varies by the tax credit they are eligible for. But they are paying their insurance company. This is a private insurance company.

Mrs. ROBY. Why is HHS not tracking this information? I mean, Congress and this committee has appropriated billions of dollars to adequately implement this law, and, you know, it is a wonder where the justification is for not having these numbers.

Secretary SEBELIUS. We do not have accurate information now because these are just being gathered. Insurers have more accurate information. The last number I heard was a comment by a couple of insurance leaders saying 80 to 85 percent of their newest customers have paid premiums in the month of January. Whether that is accurate or not, I cannot tell you. But we will get you timely information, accurate information, as soon as we have it. But customers don't pay us. They are customers of insurance companies, not of the Federal Government.

Mrs. ROBY. Yield back.

Mr. KINGSTON. Mr. Stewart.

Mr. STEWART. Mr. Chairman, I am sure you have heard the phrase that Congress is kind of like Hollywood for ugly people. You can see why I am nervous with the proximity of the camera in that case.

Secretary SEBELIUS. I thought they were doing a dental exam or something. That is awfully close. You know, I would have him back up a little.

Mr. STEWART. Madam Secretary, thanks for being here this morning. I have some questions. I would like to go quickly if I could.

MARKETPLACE ENROLLMENT

In Ms. Roby's questions and your response to her, you indicated that you don't have information on some things, and one thing that we do know from the industry, not from the administration, is that something like 25 to 27 percent of the new enrollees through the exchanges previously did not have insurance. Three quarters of them were insured before. Do you find that an acceptable figure?

Secretary SEBELIUS. I am thrilled that people are continuing to have coverage and that new folks are coming in. My understanding is that those numbers are changing over time. But, again, the insurance companies are the best validator. I think what you are quoting may be a month or so old, so that newer numbers may look different but, again, the industry has those numbers.

Mr. STEWART. We have to reason to believe though that that information, though, being a few weeks old is less accurate.

Secretary SEBELIUS. I am not talking about accuracy. I am just saying that it is changing over time, that some of the new uninsured individuals are coming in in larger numbers.

Mr. STEWART. Do you think it is going to be higher than 27 percent?

Secretary SEBELIUS. I think that is very likely.

Mr. STEWART. Okay. Do you have a figure how much higher?

Secretary SEBELIUS. I really don't, sir. What we know is that a lot of people who have never had coverage before are taking a lot longer to find out about the plan, to learn about their options.

Mr. STEWART. We don't know, though. It may be less than that figure. It may be that we end up with less than that?

Secretary SEBELIUS. I just said I don't know.

Mr. STEWART. It seems to me this has been a terribly disruptive and a very infringing program to relieve what we know is going to be 30,000,000 Americans without health insurance 10 years from now. It seems to me it is kind of like having a sliver under your nail and you cut off your finger to alleviate that. This has been such a disruptive and such a painful process, which will still leave tens of millions of Americans uninsured. And the majority of them who are enrolling had insurance previously.

Secretary SEBELIUS. Congressman, the individual market, which is what the new Marketplace is looking at, as well as people who didn't have insurance coverage, was about 5 percent of insured Americans, 5 percent. And there definitely are people in that individual market who now have new plans and new policies, many at far lower rates than they had before because they were locked into an old plan. There also are uninsured individuals coming into that Marketplace, but we are talking about the vast majority of insured Americans whose plans were not impacted or affected, except for more consumer protections than they have had before.

Mr. STEWART. Once again, Madam Secretary, you make my point; 5 percent of these people, and yet this has been a terribly disruptive program for many, many Americans.

Secretary SEBELIUS. I am saying those are the 5 percent. The formerly insured individuals who were in this marketplace are 5 percent of the insured number in America. The rest of the people who had insurance coverage were in employer-based coverage, in Medi-

care, Medicaid, in large group plans, and those have not been impacted.

Mr. STEWART. I understand, but the point still being only a small percentage of these who have joined in the exchanges were uninsured before, and we will leave 30,000,000 Americans without insurance at the end of 10 years.

If I could move on now, would you agree, and it seems like it is not just the foundation, it is the very foundation of this law, is the individual mandate, and without that mandate, the entire bill will fail, and yet—let me elaborate on that a little bit. That helps explain why the President has consistently said that he would veto even a bipartisan bill that delayed the mandate for any reason or in any way. And yet, in the last few weeks, the administration has essentially done that. They have delayed requirements that millions of Americans purchase health insurance or pay a penalty. They have essentially waived the individual mandate for the last few years.

It seems to me this would be something that the American people would need to know. And I would ask you when you made that decision, did you hold any press conferences to announce that delay?

HARDSHIP EXEMPTION

Secretary SEBELIUS. Sir, I assume you are talking about the issue where people who have coverage currently who find their coverage changes to be unaffordable could qualify for a hardship exemption and then be able to purchase catastrophic coverage. What we are trying to do is keep people in the market, not have them leave the market, and that seemed like a logical step for people who already had coverage to keep that coverage.

The hardship exemption has always been part of the law. The change was to say that if you had a hardship exemption because your new coverage was simply unaffordable, you also would qualify.

Mr. KINGSTON. The gentleman's time has expired, but let me yield time to you to answer the question on my time.

Excuse me?

Secretary SEBELIUS. That was my answer, sir.

Mr. KINGSTON. Let me make sure I understand that, though, because there was this news coverage that you could actually apply for a hardship if you lost your current policy and were unable to enroll, or it was too complicated to enroll. Is that a myth?

Secretary SEBELIUS. No, no, no. There are nine categories that were spelled out, eight or nine, in the law itself. The hardship exemption has always been there, and it has been really aimed at people who could not afford coverage, one way or the other, that they would be exempted from the mandate. There are a series of situations spelled out and then a category that basically gave some broad discretion.

As part of the transition into the new Marketplace, we said that if you are in the individual market right now and cannot afford the new coverage, not only can you apply for the hardship exemption, which was always the case, but you could qualify to purchase—because you clearly want insurance, you have been insuring yourself, you have been in the market—you could qualify to purchase a cata-

strophic policy, which had a fairly narrow group of people. You had to be under 30, basically. So that was the change in the policy; not the hardship exemption, but the fact that they could qualify to buy catastrophic coverage to keep them in the market.

Mr. KINGSTON. Okay. So it is still financially driven, not convenience-driven as the reports are?

Secretary SEBELIUS. Yes, sir. That is for the vast majority of cases. There were situations where because of the technical issues in a State or two, and people were documented as attempting to purchase coverage and couldn't get into the site, they will be eligible for retroactive coverage based on their attempt to buy coverage. But it is really the case where it is a financial issue. The hardship exemption has always been there. We said that, for instance, if you live in a State that has chosen not to expand Medicaid and you would be Medicaid eligible, you are not liable for the individual penalty because you are kind of out of luck if the State doesn't move forward on Medicaid expansion, so we have those categories. But they have always been part of the law.

STATE MARKETPLACES

Mr. KINGSTON. Okay. Let me ask you this, and maybe it would be useful for us to understand which States would fall into that category, but kind of moving on in a little slightly different direction, getting back to Dr. Harris' question about Maryland.

Maryland had such a disastrous rollout, but I understand that Oregon, Hawaii, and Minnesota, there have also been some enrollment problems. What kind of rehab do you have for those States? What kind of penalties? Do you just take over it? Because those Maryland numbers are unbelievable. I don't know how you could mess something up more than Maryland did. But I would certainly like to know that a State is, there is some sort of penalty or rehab that would apply.

Secretary SEBELIUS. Well, as I indicated, Chairman, we have a regular financial audit that takes place. The portion of the Affordable Care Act that allows the State-based Marketplaces has a funding stream, but we set up at the point that they put together plans, there is no question that some of these websites have been flawed and had more severe problems than the Federal website had. We will look very carefully at any IT money going out. It is screened and qualified. We want these sites to work.

You know, we want the Federal site to work. It took us 8 weeks of additional work to get it fixed. It was not ready as promised on October 1. It was very functional by December 1. But that work is underway in the States across the country, but we are restricting the IT funding. It goes out under audit. Whether or not the State then recovers funding from their contractors, repays the money, that is still an ongoing discussion.

Mr. KINGSTON. Did anybody get fired over the rollout? Were there vendors who were totally eliminated from future bidding processes?

Secretary SEBELIUS. I can't tell you what has happened at the State level. That is a State-by-State choice. I know some of them have changed their vendors. I think a couple are suing their vendors.

At the Federal level, we made a series of decisions. We changed overall day-to-day management immediately at CMS. We have changed contractors at CMS. We have—we are in the process of hiring a full-time risk officer. We have asked the IG to become engaged and involved, which he has agreed to do, with his team and looking at all of the contractor issues, so we have taken a variety of steps.

Mr. KINGSTON. Thank you.

My time has expired.

Ms. Lowey, we are very happy to have you with us.

Mrs. LOWEY. Well, thank you so much, Chairman Kingston, Ranking Member DeLauro.

And welcome, Secretary Sebelius.

I do apologize for arriving late. I was in a meeting with the Ukrainian Prime Minister, and then there is another hearing across the hall with Secretary Hagel and General Dempsey, so we are very busy today, but I was looking forward to seeing you.

As so many of us would agree, the Department of Health and Human Services is so very important, and the responsibility for administering some of the key services and initiatives, from early childhood education to seniors' nutrition, I strongly believe that this committee must increase investments in those areas to grow our economy and improve the quality of life of all Americans.

NIH FUNDING

One of my top priorities is to increase investments in the National Institutes of Health. Not only does NIH's work lead to future improvements in quality of life and other benefits stemming from basic research when it comes to diseases and disorders, such as autism, Alzheimer's, cancer, diabetes, food allergies, as well as the brain and big data initiatives, it is also an economic engine, and we have to remember that. Scientists in New York, for example, receive roughly \$2,000,000,000 annually in the NIH grants, and every dollar of which generates \$2.21 in economic activity. This is particularly important as there is a government-wide innovation deficit due to our inability to maintain adequate investments in research and development. And in the last 10 years, U.S. expenditures as a share of economic output have remained nearly constant while China's has increased by nearly 90 percent; South Korea nearly 50 percent. We cannot afford flat budgets that hamper innovation. It is imperative that we increase investments at the NIH.

I also strongly support proposed increases to help and protect the most vulnerable, including Head Start, the Child Care Development Block Grant, health care fraud and abuse control, and to bolster safety and preparedness.

I also believe it is important that we adequately invest in pandemic influenza bioshield BARDA, hospital preparedness, and injury and violence prevention, including gun violence.

That said, I have significant concerns with a number of proposed reductions, including CDC, LIHEAP, Children's Hospital, GME, Community Development Block Grant, and the Office on Women's Health and too many other vital initiatives would receive stagnant funding, in some cases below the levels where they were just 2 years ago, including family planning.

Madam Secretary, your Department has wide-ranging responsibilities. I will do everything I can to ensure you have adequate resources when the committee writes its fiscal year 2015 bill. And I would like to ask one question. The 2014 bill restored some of the damage done to NIH by sequestration, but as you know, we were not able to fully replace those cuts and instead fell short by a little more than \$700,000,000. Meanwhile, your budget for fiscal year 2014 includes a small increase of \$200,000,000, but in my mind, this is far below the level that we need for biomedical research.

And I just want to add, Mr. Chairman, I was privileged to attend a dinner for Research America last night. John Porter spoke. I was on this committee, as was my colleague, when he doubled the investment in the NIH. His leadership, a member of the Republican party, the chair of this committee, made an extraordinary change. And when you heard these people with a whole range of illnesses speak and talk about what investments in the NIH have done for their families and other families, it makes me proud to sit on this committee, to be part of the NIH advocacy group.

So maybe you can share with us, for some who may not be as involved in the NIH and don't interact with some of the people whose lives have been saved, what research activities could NIH pursue if additional resources were available?

Secretary SEBELIUS. Well, Congresswoman, I think that this Administration certainly shares your view that now is the absolute worst time to back away from investments in research and that NIH, which is the gold standard of the world in research, needs to be more adequately funded.

One of the reasons that the President, I think, put forward the Opportunity Growth and Security Initiative, and with a series of pay-fors, saying this is where we should be, NIH has a huge portion of that investment. But the kinds of things that are going on with the BRAIN research project, the new accelerations for cures that are underway, and the pharmaceutical collaboration with the scientists is all breathtaking.

Mr. HARRIS [presiding]. Thank you very much, Madam Secretary. Mrs. LOWEY. Thank you very much.

PHS EVALUATION TAP

Mr. HARRIS. I would hope you would join me in saying some of that increased TAP money should just be turned back over to the NIH. Maybe return that \$150,000,000 over there and increase that 211 to 350.

Anyway, Ms. DeLauro is recognized for 5 minutes for a second round.

Ms. DELAURO. Thank you very much, Mr. Chairman.

Just to tell you that the TAP effort is directed by the Congress, and particularly the TAP—it was the Bush administration that wanted to bring the TAP number to 2 percent; 2006, it went to 2.4 percent. We are 2.5 percent, and that is all directed through the Appropriations Committee. I would concur that the difference between 2.5 and 3, that is what is being proposed, but, please, people should understand where that evaluation TAP restriction comes from and where the responsibility lies. This Appropriations Committee needs to deal with that.

Madam Secretary, let me ask you this question. How many people will be insured by the end of the decade? What is the anticipation that you think you would be able to insure?

Secretary SEBELIUS. By at the end of the decade?

Ms. DELAURO. By the end of the decade, yeah.

MEDICAID EXPANSION

Secretary SEBELIUS. Congresswoman, I don't know. I can't give you that number. What I am hoping is that many more States will join the 32 Governors who are in the process of expanding Medicaid, which is a huge opportunity for lower-income working adults.

Ms. DELAURO. Which, quite frankly, they have decided they didn't want to do for whatever reasons they didn't want to do that to expand coverage for people.

Secretary SEBELIUS. That is correct, but we do have 32 Governors, Republicans and Democrats, moving ahead. We know that the recent Gallup survey indicates that the rate of uninsurance in this country as of last month is already going down, and it has gone down to the tune of they think 3,000,000 to 4,000,000 people. That is very good news. And I think that will continue. I think we will make a very serious dent in the so-called uninsured gap that currently is there because most people wanted insurance. They just couldn't afford the coverage.

AFFORDABLE CARE ACT

Ms. DELAURO. Which leads me to this comment that I want to make, given the conversation that we have listened to today. I think people sit here and believe that it was the halcyon days of health care that we had experienced before the Affordable Care Act. Let's just revisit. The health care system was failing people every year. Health care costs skyrocketed. Small businesses priced out of the market. Employers asking for higher contributions in copays and then dropping coverage. People with preexisting conditions who were being socked or left on their own. Every single year, more people had no insurance whatsoever. Annual and lifetime limits on coverage that could lead to catastrophic expenses in the case of serious illnesses. Premiums that varied widely based on factors of age, gender, location and health status. You could be cancelled in a nanosecond for treatment that you were undergoing at that particular time.

It sounds to me like some of the commentary here this morning is about taking us back, taking us back to what was regarded as a failing health care system, when people were not guaranteed coverage for maternity, pediatric care, hospitalizations, families who, as I said, faced annual caps. It takes us back to a health insurance market that rejects people with a preexisting condition, and they could be denied preventative services because they couldn't pay for it.

What are we talking about here? The American people do not want to see the Affordable Care Act repealed. They want fixes to be made, but let us look at together in the way, in the bipartisan way that this committee came together around research under a John Porter or a Ralph Regula that come together to say, how do we fix the problem so that, in fact, we can ensure people the oppor-

tunity for some for the very first time in their lives, first time in their lives, to have insurance coverage or not being told that their kid couldn't get insurance because they had asthma or were autistic? Let's focus on what the direction of this country ought to be in providing affordable health care coverage for this Nation.

That is what our moral responsibility and our obligation is to do instead of carping. Let's not carp but fix together what we can do for the benefit of the people that we represent. It is not about you and me, and I say that to my colleagues on both sides of the aisle. It is about the people who are not in the Chamber. These are the people we came to represent. That is what the Affordable Care Act is doing, is to represent the people of this country.

I yield back.

Mr. KINGSTON. Mr. Fleischmann.

OPEN ENROLLMENT

Mr. FLEISCHMANN. Madam Secretary, thank you for confirming that you will not extend the open enrollment period for individuals the way that you all delayed Obamacare requirements for businesses.

Will you delay the penalties under the individual mandate to give the people of the Third District of Tennessee and across this great Nation the same reprieve that you have given businesses? If you are considering delaying the penalties, what is your legal authority to do so?

Secretary SEBELIUS. The penalty will be applied for people who can afford insurance coverage who choose not to sign up during open enrollment. They will be liable for a penalty when taxes are due in 2015, as is stated in the law.

Mr. FLEISCHMANN. Okay. Let me say this then. I want a clarification. If an individual does not select and pay for a plan by March 31, will they or will they not be assessed a penalty?

Secretary SEBELIUS. Sir, as it states in the law, they will be assessed a penalty when their taxes are due in 2015. That is what the law says, and that is what will happen.

Mr. FLEISCHMANN. Thank you.

AFFORDABLE CARE ACT

In my remaining time, I would like to respectfully respond to my colleague's last response and remarks, and I appreciate my distinguished colleague's passion about this issue. I also have a passion about this issue. I was not in this great austere body when Obamacare was passed. I was elected in 2010. But I will say this. We did need health care reform in this country. There is no question about that, but we did not need Obamacare. One-sixth of our economy taken over by big government. In all due respect, I run into people all the time—

Ms. DELAURO. Will the gentleman yield?

Mr. FLEISCHMANN. No, I will not yield at this time. People come up to me all the time, higher deductibles, higher premiums, more Federal control. This is not good health care reform.

Yes, we could have, we could have dealt with the issue of pre-existing conditions. We could have dealt with some of the provisions that most people approve, keeping children on your insurance

until age 26. But we did not need, Ms. DeLauro, we did not need—

Ms. DELAURO. Would the gentlemen yield.

Mr. FLEISCHMANN. I will not yield.

Ms. DELAURO. You are calling me into question, and I am listening to your rhetoric.

Mr. FLEISCHMANN. I call Obamacare into question. I call this administration into question, and I call the disaster that this has caused in terms of job creation, in terms of an overreach of government authority, in terms of an administration that appears to just basically piecemeal choose what they want to delay and not delay.

So, Ms. DeLauro, I will stand with the people of the great Third District of Tennessee and this Nation to look for other alternatives other than Obamacare to solve this nightmare.

Thank you. I yield back.

Ms. DELAURO. It is unfortunate for the debate on Affordable Health care that the other side did not come forward with any program.

Mr. FLEISCHMANN. I yield to the chairman.

Mr. HARRIS. Thank you very much.

No, actually, I guess of the members on this side of the aisle, none of us were here. But our understanding was—and I applaud you—on this side, none of us were here when this bill was passed—and I applaud you for calling for bipartisan. But I also urge you to remember one of the reasons why this bill is failing is because it was not passed with bipartisan efforts.

Madam Secretary, I am going to follow up in the balance of Mr. Fleischmann's time. On that question of the hardship exceptions, is the Wall Street Journal report from Monday morning incorrect in fact that all an individual needs to do is just to claim that they feel that the insurance policy is unaffordable and they will get a hardship exemption? Is it incorrect, their research? I am sure you have seen it.

Secretary SEBELIUS. Sir, what we said—

Mr. HARRIS. Madam Secretary, are they correct or not?

Secretary SEBELIUS. If you had a policy—

Mr. HARRIS. No. I am not talking about someone who didn't have a policy. That is not what the editorial said. The editorial said someone who didn't have a policy.

Secretary SEBELIUS. The editorial was incorrect. It applied specifically to people who were in the market who found their new policy unaffordable so that they could qualify. These were people who were insured, who wanted to keep insurance—

Mr. HARRIS. So their editorial was wrong? Is that correct? In that rule, someone will not get a hardship, but you said that if you don't have a policy, in response to Mr. Fleischmann, that if they can afford it, they will get a penalty.

Madam Secretary, where is the definition of who can afford a policy? I never met someone who said they thought their policy was affordable. Where is the definition? You said, and I quote you, if they can afford it. What is the definition of "affording"? And my time is up, so you can answer on my time.

Secretary SEBELIUS. I would be happy to send you the rule as it stands, and I am—

Mr. HARRIS. You are going to have to answer that for me.

Secretary SEBELIUS. This was specifically dealing with people who were in—

Mr. HARRIS. Ms. Roybal-Allard.

Secretary SEBELIUS. Dr. Harris, could I answer your question?

Mr. HARRIS. No, you will have your chance my round. When it comes up, you can finish answering. Thank you, Madam Secretary.

CDC FUNDING

Ms. ROYBAL-ALLARD. Secretary Sebelius, each year, the Department's annual budget request for CDC is smaller than the year before. For example, the President's budget request to Congress for fiscal year 2011 proposed a core budget for the CDC of \$7,600,000,000. That was a billion dollars more than the \$6,600,000,000 that you request today, even before adding losses for inflation. This 14 percent drop in the CDC's request over the last 3 years is actually stunning, and it seems to me that the CDC continues to at disproportionately suffer from cuts compared to other HHS agencies. In fact, this year's budget request put CDC's budget authority at levels lower than 2003 and includes mandatory prevention money allocated to the CDC from the Affordable Care Act that was intended to do more for the prevention than the core CDC activities.

Given that State and local health departments rely on CDC funding to ensure adequate childhood immunization rates, develop capacity to respond effectively to public health emergencies and to build capabilities to track environmental hazards, and given the fact that we are seeing old and new and sometimes mysterious diseases starting to pop up throughout the country, how will CDC continue to support these critical core public health functions with the cuts proposed in your budgets? And what has been the impact of these cuts on CDC's prevention investments over the past 4 years, especially when you consider that the public health fund mandatory spending was intended to supplement prevention efforts, not to supplant the CDC core budget?

Secretary SEBELIUS. Well, Congresswoman, I would say a couple of things. Our discretionary budget for 2015 is over a billion dollars smaller than the discretionary budget target for 2014. So we start with the fact that we have to allocate funds in a reduced budget environment, and we certainly share the concern, as I can assure you so does Dr. Frieden, that the efforts of the Centers for Disease Control and Prevention is not only critically important as the backbone of public health but is increasingly important in our global health initiative. So we have tried to focus the budget on the areas that he feels are top priorities.

Some of the program eliminations were due to duplication of programs in other areas. Some are able to be reduced because we anticipate that more people will have health coverage to pay for services that an independent program would have paid for in the past. And the services will go on; we just don't need the money through CDC.

But I am pleased that this budget does focus on things like increasing efforts in antimicrobial resistance, which is a huge health fear throughout the health system in the United States and

throughout the world. And the CDC has incredible expertise. We are launching a new global health security initiative, which actually keeps the United States citizens safer and more secure to build capacity for surveillance detection. Again, those are areas that Dr. Frieden wants to focus new funding, and so he has chosen to reduce some of the funding allocations.

I would finally say that you are absolutely right, that the Prevention Fund was to not supplant old efforts but to enhance prevention efforts. I am pleased to tell you that given Congress' activity in 2014, those efforts will be dedicated to prevention efforts, not used to backfill programs. And I think that we would see that going forward in 2015. We have, again, not suggested that the Prevention Fund be used to supplant CDC funding.

Ms. ROYBAL-ALLARD. So are you saying that these cuts have had no negative impact then on public health efforts?

Secretary SEBELIUS. I would say that all cuts have negative impact, and certainly we saw during the shutdown the very difficult time that a lot of public—you saw the real impact that CDC had when they had to ask employees to stand down, and that sent shock waves through States throughout the country. So every cut has an impact, but we, as I say, have a billion-plus dollars less to work with in 2015, and we are trying to be very strategic about how those funds are moving forward.

Mr. HARRIS. Thank you very much.

Before I start my question, I am just going to comment. You know, we have still I think five members remaining in the second round. I know your time is valuable, so we have got to keep it to exactly five minutes so that we respect your time and your limitation here.

HARDSHIP EXEMPTION

So let me go ahead and let you answer the question that I interrupted your answer before for time purposes. It has to be very specific. When you said that if they can afford it, someone who has never been insured before will get the penalty on this year's taxes. That is what you said, if they can afford it.

Secretary SEBELIUS. In 2015—

Mr. HARRIS. Correct.

Secretary SEBELIUS. The fee will apply.

Mr. HARRIS. And who decides if they can afford it?

Secretary SEBELIUS. There is a hardship exemption. If you self attest—

Mr. HARRIS. So self attest—so the Wall Street Journal was right on track then?

Secretary SEBELIUS. Sir, there are criteria in the bill. There are nine categories.

Mr. HARRIS. I understand it. That is exactly what the Wall Street Journal said, self attestation.

Secretary SEBELIUS. There are nine categories.

Mr. HARRIS. Now, Madam Secretary, let's talk a little bit about some—first, by the way, I just want to make a comment. I hope that you have the same enthusiasm for a non-medical-marijuana-free generation that you have for a tobacco-free generation. As you know, the Attorney General has decided not to pursue, not to en-

force the Controlled Substance Act in two States. And I hope you agree with me that marijuana is at least as dangerous as tobacco.

ABORTION

But let me go ahead and talk a little bit about some transparency because I think Mr. Shimkus asked you back in December about the transparency of whether or not you can determine whether a plan on the exchange covers abortion. You said all you have to go is to the summary of benefits to see that. These are the summary of benefits of every exchange plan in Maryland. None of them have an indication whether they cover abortion in the summary of benefits. It is not transparent whatsoever. I don't know if this is true in any other State. All I know is, in my State, you can't do it. Do you believe it should be fully transparent to the person visit being the Web site?

Secretary SEBELIUS. I think it should be transparent. All the benefits are.

Mr. HARRIS. Thank you very much. Now, and just so you know, it is not transparent. So when you look at these Web sites and provide oversight, please make sure, and this is not just from us. I mean, Planned Parenthood of Northwest has made the same complaint. It is very obscure. You can't tell.

Secretary SEBELIUS. I understand. I think people should know what the benefits are.

Mr. HARRIS. We are going to agree. You see. We are going to agree on something.

Now, let's go ahead a little further, though, and talk about the billing because the Maryland insurance commissioner, she has said that, in fact, the insurance companies don't have to invoice separately for abortion coverage and non-abortion coverage in plans that cover abortion. I mean, literally she has said that. I assume that the plain reading of the law that says there has to be a separate charge means that you actually have to be able to determine a separate amount somewhere? Is that correct? Is that your interpretation of the—

Secretary SEBELIUS. Again, I can't speak to what the Maryland Insurance Commissioner said or didn't say, but I can assure you we will follow the law in the Federal Marketplace.

Mr. HARRIS. But you actually have to oversee all the exchanges, don't you, not just the Federal marketplace, don't you?

Secretary SEBELIUS. Well, we have supervisory—

Mr. HARRIS. Correct. You do.

Secretary SEBELIUS. No. Sir, the States run their marketplaces. They qualify the plans, but they have to follow the law also.

Mr. HARRIS. Correct. If, in fact, the Maryland commissioner, if, in fact, that is her determination, that there doesn't have to be even a separate invoicing of it; that that would, in fact, be a violation of the law.

Secretary SEBELIUS. I would be happy to take a look at what she said and follow up with our team.

MARKETPLACE ENROLLMENT

Mr. HARRIS. I hope you do because, again, the reading of the law is pretty plain to me.

Now, let me ask you a question because this was brought to my attention by an insurance agent in my State. April 1 comes, April 10, somebody sees a rerun of the, you know, "Between Two Ferns" deal, and a young healthy patient says, you know, what, the President convinced me; I want to go out, and I want to be insured. Healthy young person, exactly the kind of person we want on our exchanges. They can't, can they, in most States?

Secretary SEBELIUS. As any open enrollment, they need to wait until November 15 when it opens again.

Mr. HARRIS. So we designed a system where that healthy 25-year-old who wants to buy insurance on April 15 can't buy insurance. Now, that is different, the system before—and I agree with the ranking member, there was plenty wrong with the system before. But I will tell you that on April 15, a healthy young person could have gone out and bought a policy. So when that healthy young person, God forbid, has an accident in August—let me follow up—the individual market, and ranking member suggests go to the individual market—those policies can't be purchased, unless you have a qualifying event, those policies can't be purchased, can they?

Secretary SEBELIUS. They can. Absolutely.

Mr. HARRIS. They can be purchased where?

Secretary SEBELIUS. They can be purchased outside the Marketplace in the individual market. They cannot qualify for a tax credit until the next open enrollment season, but they can purchase an insurance policy at any time. They just can't use the open enrollment through the Marketplace and qualify for a tax credit.

Mr. HARRIS. And, Madam Secretary, in Maryland, it is not available. In Connecticut, only one plan.

Secretary SEBELIUS. What is not available, sir?

Mr. HARRIS. That purchase of that policy for the individual—

Secretary SEBELIUS. I don't think that is correct. I think in every State, there are individual policies sold inside and outside the Marketplace.

Mr. HARRIS. Mrs. Lowey.

Secretary SEBELIUS. Dr. Harris, if I might take a moment, I want to correct a statement I made earlier to, even though Congressman Joyce is not here. I have been told I misspoke. And I did that unintentionally. The SHOP data enrollment is not in the current enrollment report, but I will give him the information that we have and send that up today, but I just wanted to make that clear.

Mr. HARRIS. Thank you very much.

Mrs. Lowey.

Mrs. LOWEY. Thank you, Mr. Chairman.

Before I ask the question, ranking member would like 30 seconds.

Ms. DELAURO. I thank the gentlelady. I think Mr. Fleischmann has said that we shared the concern that the health care system was failing people and that we needed to reform it. Well, I would just like to mention to you that we had 6 years of a Bush administration, where, if that was the case, nothing was done to address it. We waited a 100 years to get an Affordable Care Act passed. The majority today, as it did when those of us who were here were going through this process, the majority has no program, no plan,

except to repeal it. If you have a plan for dealing with affordable care for the people of this Nation, I would please ask you to come forward with it and just lay out what it is that you would do. I thank the gentleman.

Mr. JOYCE. Will my friend yield? Will my friend yield? But I do have a plan.

Ms. DELAURO. No. Mr. Fleischmann would not yield to me, so I have asked Mrs. Lowey if she would yield me 30 seconds.

Mrs. Lowey.

Mr. JOYCE. Unanimous consent that it won't be charged against you.

And I just want to make sure my friend, the ranking member, said number one—unfortunately, I am going back and forth between Defense, and I have got some issues across the hall, so that is why I am not here, but when I come back, I will always be happy to yield my friend time for a good spirited discussion on any subject.

Mr. HARRIS. Thank you.

Mrs. Lowey, you are up. We stopped the clock for that.

Mrs. LOWEY. Thank you so much.

And before I ask my question, again, I mentioned John Porter and how we worked together in a bipartisan way. And as with any large program, any large program, whether it is Social Security and Medicare, there have been revisions. So I do hope that we can be constructive, not only in these hearings but in other discussions. And if there are specific changes that you would like made, I do hope we can have healthy discussions to amend it. You have seen that happen as we move along.

AUTISM

So I would like to ask a question about autism. It is amazing. I go on an autism walk every year, and the numbers are just increasing tremendously. As prevalence rates have increased, and we know the rates have increased dramatically in the last 20 years. There will be increased demands for services for children and young adults. Could you share with us how the budget request helps families and individuals with autism?

Secretary SEBELIUS. Congresswoman, I would say several things. One is that if a family has a child or even an adult with autism, that individual will now qualify for health insurance, where they may not have in the past if that family was in the individual market. That is a big step forward just in terms of underlying care and being able to access it. There is some very important research underway at the National Institutes of Health. Autism is also one of the areas in the brain mapping structure that will be focused on by the NIH, and I think that bears very promising resources.

We also have an autism working group coming up with a series of strategies and suggestions, everything from hospice care and home-based relief care to strategies about community events and enhanced research that is part of the ongoing autism effort. So I would say there is basic underlying care. There is additional research. There is additional funding. But, again, I think the need is huge in this area. And as more identification takes place of who all is in the autism spectrum, those numbers grow.

Mrs. LOWEY. So, in addition to the research, I do hope we can get together, whether it is HRSA, the Institute of Community Living, there is a lot that we have to do.

Is my time up? No? Okay.

Mr. KINGSTON. Fifteen seconds.

CDC FOOD ALLERGY GUIDELINES

Mrs. LOWEY. Let me just say in the 15 seconds, I have been working on food allergies, celiac disease, for a long time. And I probably don't have time, but I hope you can share with me at another time CDC's efforts to disseminate the voluntary guidelines to schools and the Department efforts because it is a really important issue.

Secretary SEBELIUS. Be happy to do that.

Mrs. LOWEY. Child has an anaphylactic reaction, too often too many people don't know what to do.

Thank you, Mr. Chairman.

Mr. HARRIS. Thank you.

Mrs. Roby.

ABORTION

Mrs. ROBY. Thank you. Just a quick follow up on my first line of questioning. There was an article that came out today, the insurance industry takes issue with the administration's claim that you do not have the information that I asked and that you testified about yesterday. The insurance industry says that they have a lot more information than they are letting on, and they have real hard data about the percent that it paid. And if the administration and if they have not processed those yet and compiled the data, that is a choice they are making, but they have the data. So the insurance industry obviously takes a differing position than that of this administration.

But I have short time, and I have got another important line of questioning that I want to ask. According to Paul Bedard writing in the Washington Examiner last week, and I quote, "Planned Parenthood is going to bat for the White House, hosting more than 500 events in 18 cities to get Americans into the health insurance system. In eight States, officials from the women's health and abortion provider will go to grocery stores and even indoor soccer fields to enroll people. It also plans to dispatch 500 canvassers and knock on 25,000 doors." Earlier this year, the New York Times reported that Planned Parenthood is one of the most aggressive groups, going door to door to enroll individuals into Obamacare. And last year, we learned that Planned Parenthood affiliates received at least 655,000 in navigator funds to help with Obamacare enrollment. And I am sure you know Planned Parenthood is the largest abortion provider in the Nation, carrying out more than a quarter of all abortions in this country.

So I would like for you to tell us, has the 500 events, 18 city Planned Parenthood enrollment initiative that I mentioned received Federal funds? And what is the total amount of Obamacare-related funding that has been directed to Planned Parenthood or its affiliates either directly or as a subgrantee or subcontractor?

Secretary SEBELIUS. Congresswoman, I can get you the information on the Navigator grants. I don't have that State by State. Those were competitive grants of known community organizations, and I am happy to provide that.

The activities that you talk about are ones that I assume the organization is conducting. They have no funding from the Federal Government. I do think that Planned Parenthood is a provider of preventative care across this country. Over 6,000,000 people get cancer screenings and well woman checkups and contraception—

Mrs. ROBY. Could you please for this committee, because this is a very important issue, could you please provide for us at some date certain, you know, by end of next week and guarantee us that you will give us an itemized list of each grant or contract that has been made available to Planned Parenthood or a Planned Parenthood affiliate under Obamacare?

Secretary SEBELIUS. I can give you the Navigator grants, which as I said, were competitive grants in communities across this country.

Mrs. ROBY. If you can give that to us, that would be helpful.

HEALTH INSURANCE COVERAGE

Secretary SEBELIUS. Yes, ma'am.

Mrs. ROBY. According to April 12, 2010, edition of the New York Times, President Obama agreed to volunteer to enroll in a health insurance exchange. For the record, will you tell us what political appointees at HHS have enrolled in the health insurance exchanges?

Secretary SEBELIUS. Well, I think you will be pleased to know that I don't access people's personal information, and so I cannot give you that information, nor will I ask anybody about their information. Their employee coverage is paid for as part of the Federal plan. The Marketplaces are for people who don't have coverage in the employer market.

Mrs. ROBY. The point is, Madam Secretary—

Secretary SEBELIUS. If you have affordable employee coverage, I assume they will stay where they are.

Mrs. ROBY. The point is this, if it is good enough for the American people, it should be good enough for political appointees.

Secretary SEBELIUS. It isn't about political appointees. It is people who don't have affordable employer coverage. Federal employees, State employees, have affordable employer coverage, and they would not access the Marketplace.

Mrs. ROBY. We today have spent time, and we have differing opinions, obviously, but we have spent time today pointing out deficiencies that we see in this law. And, again, I think it is important for the folks back home in Alabama and people all over this country to know that the Federal Government is not exempting themselves that they mandating that the American people have.

Secretary SEBELIUS. Again, no one in Alabama who works for one of the new auto factories who has an employer-based plan will be accessing this Marketplace. No one who works for city or county government will access this plan. This is for people who didn't have employer coverage, affordable employer coverage, and they now have an option.

Mr. HARRIS. Thank you.
Ms. Lee.

AFFORDABLE CARE ACT

Ms. LEE. Thank you very much, Mr. Chair.

First, let me just say to you, Mr. Chair, and others who made a comment about the Affordable Care Act not being bipartisan or receiving one Republican vote. I, during that period, chaired the Congressional Black Caucus and led many of our efforts—and, Mr. Chairman, I want to mention this—led many of the efforts on the negotiations on the Affordable Care Act.

Much to my disappointment, our leadership and the President accepted many Republican suggestions in the Affordable Care Act, one by one by one. Not one single vote on your side. So I can't sit here and let you say that you didn't get, we didn't get bipartisan support when, in fact, the President and our leadership reached out, incorporated many of your suggestions. And you all still didn't vote for it, and I was there. I saw this go down, and I think many of us understood what was taking place. And I was very disappointed, but in the spirit of compromise, we went along with it.

SEX EDUCATION

Let me say to you, Madam Secretary, I want to commend you and the administration, based on what we are seeing as it relates to HIV and AIDS and other sexually transmitted infections, as it relates to young people in terms of preventing teen pregnancy. I want to just see how does the budget reflect this ongoing commitment because I think you all are doing a really good job in developing comprehensive sex education for young people.

LIMITED ENGLISH PROFICIENT POPULATIONS

And then, secondly, I serve as co-chair of the Congressional Asian Pacific American Caucus, and of course, we have been dealing with this issue as it relates to inadequate translation services provided to limited English proficient populations, both in the translated applications, as it relates to the Affordable Care Act, the call centers, given that there are so many different languages. And so how does this budget, you know, help address the issue of linguistically appropriate services in health care and the exchanges and to get the information out and to help those who need to understand how this works.

Secretary SEBELIUS. Let me take the last part first. We share your concern that this is a very diverse and rich country. Based on its diversity and having language-appropriate information is essential. So the call center can answer questions in up to 150 languages. We have printed paper applications in five languages. That is not enough, and we are looking at what resources we might need to expand the paper application process so that we could have more printed applications available.

The website as you know, is available in Spanish and English. Again, one of the goals is to look at how culturally diverse the website could be. It would be, I can guarantee you, virtually impossible to have the Website in every language that is spoken in this

country. So having translators available, having a variety of help available is one of the things.

We are also looking at how we diversify our Navigator force on the ground, so even if you can't go to the Website in your native language, you may be able to have someone on the ground. All of those are underway. And with the resources available, we are trying to take that very seriously, gathering information from what we know now where the highest problem areas and where the markets are.

I would say in the HIV/AIDS area, you know that having a National Strategy is a first time for any Administration. It never has occurred before. We are taking that very seriously, focussing resources on the high-risk populations at high-risk and the communities at high-risk and certainly testing, treatment, and access to ongoing treatment are the three areas we are refocussing on, trying to get rid of some of the stigma around testing but also making sure that people are routinely screened and come forward and then connecting them to treatment and keeping them connected to treatment, because that is a very effective life-saving strategy.

POVERTY

Ms. LEE. And finally, and I have a few more seconds. I just want to say that this subcommittee really is the subcommittee that can address issues as it relates to poverty and income inequality. We passed several amendments to all of our bills talking about and laying out our efforts to reduce the existence of poverty. So I hope in this budget, we can see some antipoverty ladders of opportunity, provisions, and a way to really address income inequality through your agency.

Mr. HARRIS. Thank you.

Mr. Stewart.

Mr. STEWART. Yes, Mr. Chairman, and I understand you would like me to yield you 30 seconds.

Mr. HARRIS. Thank you very much for yielding 30 seconds.

AFFORDABLE CARE ACT

Madam Secretary, in response to the last answer that you can buy insurance on the individual marketplace after March 31, you ought to tell USA Today, Kaiser Health News and AARP, all publications that say you can't do it in the individual market or the exchange after March 31.

Yield the balance of that time to Mr. Stewart.

Mr. STEWART. Thank you, Mr. Chairman.

Madam Secretary, I admire your courage. I really do. I think you have this incredibly difficult task of defending this law before the American people, and I am so glad that I don't have to do that. I was a small business owner. I know how difficult it used to be and how hard we worked to provide adequate insurance for my employees, but I am telling you, this is worse. In many ways, it is much worse, and the American people know it. The health insurance program that we had before in country wasn't perfect. I don't think anyone has ever claimed that it was, but the vast majority of Americans were satisfied, which is why we told them, or the administration told them: If you want to keep your doctor, you can

keep it. If you want to keep your health insurance, you can keep it. And we now, of course, know that that is not true. Much of that turned out not to be true as well, of other specifics of this law.

I would like to talk just quickly about the innovation centers, which and I am quoting, they are created to provide innovative payment and service delivery models to reduce payment expenditures. Essentially, they are there to review and to fund grants. In 2011, there were only 68 FTEs that were involved with the innovation centers. This year, there is going to be 450, which seems like an awful lot of people to review and to, you know, streamline this process of granting these government grants. Can you assure the committee that there is—none of these employees in the innovation centers are involved in the marketplaces?

Secretary SEBELIUS. Congressman, there is no involvement of the Innovation Center in the Marketplace. There is one Innovation Center—it is a center in the agency of Centers for Medicare and Medicaid Services. It is funded through the Affordable Care Act.

And I would say, for the first time, we have a research and development arm aimed specifically—and it isn't about the Marketplace at all. It is aimed specifically at how quality can be improved and costs can be lowered for Medicare and/or Medicaid programs, the big public programs run by CMS. That is what the aim is.

I think the work that they are doing will benefit everyone, even if you are not in Medicare and Medicaid, because things like lowering hospital-acquired infections, looking at preventable readmissions, looking at elective deliveries that could cause long-term harm to infants, those kinds of efforts are very much under way. But it has nothing to do with the Marketplaces.

Mr. STEWART. Okay. And, as well, you can assure us, then, that they are not involved with the implementation activities of Obamacare?

Secretary SEBELIUS. That is correct.

Mr. STEWART. Okay. Thank you.

In recognition of the shortness of time, then, Mr. Chairman, I will yield back my last 30 seconds.

Mr. KINGSTON. Mr. Womack.

AFFORDABLE CARE ACT IMPLEMENTATION

Mr. WOMACK. Thank you, Madam Secretary, for your testimony here this morning. I apologize for being late. I am sorry, I missed a lot of it.

But at a House E&C hearing back in November, Henry Chao, the CMS Deputy CIO who oversaw technical development of the exchanges, said there were some back-office functions that were not complete yet.

And I want to go straight to an issue that is relative to my district specifically. I have the largest home of Marshallese population in the United States. Many of these individuals have Medicaid-qualifying incomes, but, as a COFA migrant, they are not Medicaid-eligible. However, they are eligible for the premium subsidy under the Affordable Care Act. But, as I understand it, their applications are being kicked back and forth between the State, which cannot provide Medicaid coverage, and the exchange, which cannot

process the applications, ultimately bouncing the applications out of the system on the back end.

And I know the Arkansas insurance commissioner has sent word to D.C. that they have this problem. So I am asking, are the back-office operations and IT support functions fully operational and secure?

Secretary SEBELIUS. Sir, we are in the process of testing, State at a time, all of the Medicaid inbound and outbound. They are built, and each State has a slightly different system. What we are finding in some States—and I can't tell you off the top of my head if Arkansas is one of them—that the State is not able to receive the automated account, so we are doing a more manual workaround.

But we have a very high priority to make sure that people who are eligible get enrolled in a timely fashion, and we will do that.

Mr. WOMACK. Can you give us a timeline?

Secretary SEBELIUS. Of enrollment?

Mr. WOMACK. When these functions will be operational, when you can get back to Jay Bradford and say—

Secretary SEBELIUS. They are operational now at the Federal level, both outbound—so if someone comes to the Federal Web site and appears to meet the State qualifications for Medicaid, they are automatically sent to the State. Some of the States can receive that automated information now. Some States do not have the capacity to do that, so they are sent a paper file application. When someone comes to the State and appears to be qualified for the Marketplace, they are referred the other way.

So the automated functions now are built. We are testing a State at a time to make sure that they can receive back and forth.

Mr. WOMACK. Would it be an unreasonable request to have somebody from your office contact the Arkansas insurance commissioner—

Secretary SEBELIUS. Not at all. I would be happy to.

Mr. WOMACK [continuing]. And close that loop?

Secretary SEBELIUS. Sure. Absolutely.

Mr. WOMACK. Okay.

MEDICARE ADVANTAGE

And then, finally—and I know I am closing and you have a hard time to be out of here—the President's budget, released last week, proposes nearly \$35 billion in additional Medicare Advantage rate cuts over 10 years. And it is a huge thing for my district, as it is for a lot of people.

Can you guarantee my constituents that they won't lose their Medicare Advantage plans?

Secretary SEBELIUS. Absolutely.

Medicare Advantage, I think, Congressman, is a very positive story with the Affordable Care Act. The allegation—and it has already been said that many of the Members were not here in 2010. The allegation was that if you pass the Affordable Care Act, you will destroy Medicare Advantage, no one will have a choice, it will be gone. Just the opposite has happened. Enrollment is up 30 percent. Premiums are down almost 10 percent. More enrollees are in

higher-quality plans than were in 2010. We have seen a very positive growth in the plan; we anticipate that will continue.

What is happening, though, is insurance companies are no longer being overpaid based on the care that they are delivering. The fees are closer right now to a fee-for-service in Medicare. And so I think that we see that progress continuing.

Mr. WOMACK. I thank the Secretary.

Thank you, Mr. Chairman.

Mr. KINGSTON [presiding]. Thank you, Mr. Womack.

Madam Secretary, we appreciate your being here today. I do want to close with a few quick question, and we can do those for the record.

And Members will have 2 weeks to submit further questions.

[The information follows:]

The transcript record states that "Members will have two weeks to submit further questions." There does not seem to be an actionable insert located here?

AFFORDABLE CARE ACT OUTREACH

Mr. KINGSTON. I am concerned about the advertising budget. I think it is about \$770 million. And yet it will be past the enrollment period, so I am not sure why it needs to be so high.

Secretary SEBELIUS. Sir, I don't really—I wish we had \$770 million of any kind, but I don't know what—

Mr. KINGSTON. It is the Consumer Information and Outreach, so maybe—

Secretary SEBELIUS. That is the office—that is not advertising.

Mr. KINGSTON. Okay.

Secretary SEBELIUS. The Consumer Information and Outreach Office is the office that actually manages the entire Marketplace program, both at the State and Federal level. Those are employees in the Marketplace functions, but that is not an advertising budget.

Mr. KINGSTON. Okay. I am still concerned as to why we need that, but, you know, we will follow up with some questions.

Secretary SEBELIUS. I would be happy to give you information—

Mr. KINGSTON. Yes.

Secretary SEBELIUS [continuing]. About what they do. That is the third big center in the—

Mr. KINGSTON. Also, Ms. McCollum and I have been critical of some of the military recruitment ads as being ineffective. And I am always suspicious when I see Federal Government ads anyhow. One that caught my eye, and, frankly, as a male, it was very offensive, as the father of two sons, the "bro" insurance ad for health care. I am not sure if that was a Federal ad or one of the State exchange ads—

Secretary SEBELIUS. It was not a Federal ad.

Mr. KINGSTON [continuing]. But it had young men doing handstands on a keg of beer.

And so I am just interested, and I will follow-up with you, in terms of how effective some of these ads are and what kind of metrics you use. Because we have found with some of the military ads, they did not have metrics that showed the effectiveness of it.

Secretary SEBELIUS. I would be happy to share with you what the CMS folks found in focus groups and others, in terms of the advertising. But the “bro” ad is not ours.

Mr. KINGSTON. Okay. Because I know there was another one that was not yours also that they had in Colorado.

The other thing, getting back to Ms. Roby’s question about political appointees and Obamacare, the President did sign up, very visibly, on December 23rd about it. And I think that what the concern is Congress has had a lot of public input on signing up Congress, signing up our staffs, subsidies for them.

And so I think the question is—and I will submit it to you—is on political appointees in general and their staffs.

I also wanted to ask you how navigators get rated, in terms of their effectiveness.

MARIJUANA

And then, switching gears—and, Rosa, I don’t know if you saw this, but Patrick Kennedy was on TV, and he is, you know, a former colleague of ours. He had, very publicly, some drug issues, drug addiction issues. And he is very concerned about marijuana.

And one of the things he said that caught my attention is that the States are getting so many tax revenues, and there is so much profit in it, and he said, yet, their natural market is going to be teenagers, and they are going to make sure that it is very attractive to teenagers.

And so, as we go through this murky water of State laws changing, contrary to Federal laws, it is something that I think your agency needs to be very concerned about.

And then, kind of getting back to what Dr. Harris said is, you know, is there a position of Health and Human Services that medical marijuana, or marijuana in general, is more harmful than tobacco?

Secretary SEBELIUS. Well, I would be happy to get you—we certainly have done a lot of research and a lot of information, and I would be happy to get that to you.

I can tell you that there certainly are harms and some long-term brain harms that go along with smoking marijuana over a period of time that have been clearly documented. There is a lot of public health information.

But I would also tell you that there is no comparison in terms of health risks for someone who is smoking tobacco and addicted to nicotine versus marijuana, in terms of cancer and death and secondhand smoke.

So there are dramatic health differences, but I would be happy to present the information.

TOBACCO

Ms. DELAURO. All I would just say with regard to that, Mr. Chairman, is—and I did a press conference on these new e-cigarettes and e-hookahs. That is an area that we ought to be trying to focus on, where the tobacco industry, the way they market it to 12-year-olds for cigarettes, because that was their new market, they are now marketing again to 12-year-olds for e-cigarettes and e-hookahs.

But the information was 480,000 people die every year from a tobacco-related illness. Now, we went to war in Afghanistan with the 3,000 and almost 4,000 people who died, and yet we continue, when we can prevent these deaths from tobacco.

Thank you.

Mr. KINGSTON. The marijuana thing is—just when I heard Patrick talk about it, it really caught my attention.

But thank you very much for your time, and thank your staff. Secretary SEBELIUS. Thank you, Mr. Chairman.

**Department of Labor, Health and Human Services and Education
and Related Agencies**

Budget Hearing with Secretary Sebelius

March 13, 2014

Questions for the Record – Chairman Kingston

1) ObamaCare FY 2014 Funding

1A) The FY 2015 budget request includes a legislative provision for \$433 million of mandatory funds to support CMS Program Management. The budget document is very vague and light on details related to this proposal. In fact the legislative text for this request is not in the budget documents.

The most descriptive narrative on these funds is in the Budget in Brief (page 55). It certainly could be interpreted that these funds will be used or could be used to implement “responsibilities assigned in the Affordable Care Act.” However, in the CMS Program Management section it does not specifically identify these funds as a resource.

Is it HHS’ expectation that these funds would be used in addition to the \$1.8 billion of ObamaCare funds described in the CMS Program Management account?

Specifically, what are these funds to be used for?

The \$433 million will be used for the following purposes:

- \$400 million represents mandatory Program Management funding that will be used to implement all the Medicare and Medicaid proposals that are included in the President’s Budget. CMS estimates the savings from these proposals to be \$414.5 billion over the next ten years. This administrative funding was requested in last year’s budget as well, as previous legislative reform packages have included funding to CMS to enable implementation of the reforms.
- \$30 million will be used annually through 2017 for a consensus-based entity focused on performance measurement. This investment will help CMS implement value based purchasing initiatives and other models, which focus on performance based payments. Last year’s budget also requested this funding.
- The remaining \$3 million represents FY 2015 funding for three new offsetting collection proposals: a Survey and Certification Revisit Fee, administrative fees to offset costs incurred for the Federal Payment Levy Program, and the retention of a

portion of Home Health Agency (HHA) Civil Monetary Penalties. CMS is reinvesting those funds to improve the quality of care of patients receiving home health services for quality improvements.

1B) What is the current assumption of ObamaCare related Offsetting Collections for CMS Program Management in FY 2013, 2014, and FY 2015 given new knowledge on enrollment since the budget was released? Please describe what these funds were used for in FY 2013 and projected for FY 2014 and FY 2015.

Our assumptions for Federally-facilitated Marketplace user fees at the time the FY 2015 President's Budget was submitted were \$200 million in FY 2014 and \$1.2 billion in FY 2015. User fees for the Federally-facilitated Marketplace were first collected in January 2014 to align with the first month of Marketplace coverage. We have not yet updated user fee projections for FY 2014 and FY 2015, as we expect many individuals will enroll in Marketplace plans through the end of this month, and thus do not have final enrollment numbers.

1C) What is the current estimate from all sources that CMS will use to support ObamaCare activity in FY 2014 and FY 2015 (include the FY 2013 actual for each source)? Please provide an itemized list of activities with the cost, source of funds, and amount of funds obligated or planned for each year? Please ensure it includes funds transferred in from other agencies, offsetting collections, etc (all sources).

Page 349 of the CMS Congressional Justification lists all sources expected to support CMS Marketplace operations in FY 2013, FY 2014 and FY 2015.

Marketplace Spending by Activity (in millions)

| | FY 2013 | FY 2015 |
|--------------------------|----------------|----------------|
| Marketplace Operations | \$371 | \$770 |
| Consumer Info & Outreach | \$734 | \$774 |
| IT | \$374 | \$201 |
| Federal Administration | \$68 | \$85 |
| TOTAL | \$1,545 | \$1,829 |

The FY 2014 Marketplace Plan is still under development.

1D) Please provide a breakdown of the total amount of CMS Program Management Funds to be used to support ObamaCare Activity in FY 2013, 2014, and 2015, with a breakdown of the activities supported and amount per year.

Total CMS obligations from Program Management in FY 2013 were \$520 million. The approximate break down per activity was \$68 million on Federal Administration costs, \$330 million on consumer outreach activities such as the call center, \$20 million on eligibility and enrollment activities, \$20 million on plan management activities, \$55 million on information technology activities, and the rest on activities to support the Federally-facilitated SHOP, payment management functions and technical assistance to stakeholders.

For FY 2015, Program Management funding for Marketplaces will be used to support enrollment, outreach, and education activities. The FY 2014 Marketplace Plan is still under development. The FY 2015 President's Budget proposes \$629 million in Program Management funds for CMS Marketplace costs.

1E) What are the assumption used to develop the FY 2015 budget request for ObamaCare in FY 2015, to included enrollment, participating providers, states using the federal exchanges, and other key assumptions. Please provide the specific number and attribute for each as well as how it calculated in to the total request. Further, provide the FY 2013 and FY 2014 data for each FY 2015 assumption.

CMS currently performs Marketplace functions for the individual market in 36 states, and we expect a similar level of participation in FY 2015. Consistent with projections by CBO, we anticipate that enrollment will increase in 2015, and note that CBO anticipates approximately 13 million individuals will be enrolled in both the state and Federal Marketplace in 2015. Although many of the Federally-facilitated Marketplace operations are workload-driven, there is not a one-to-one impact of increased enrollment on workload and therefore costs. The FY 2015 request represents an assessment of needs based on the costs of existing operations, as well as new functions that will come online in that year such as reinsurance, risk adjustment, and risk corridors.

1F) What is the projected impact on public health, prevention, bio-medical research, and mental health training based on your decisions to siphon off additional funds in FY 2014 for ObamaCare through the Secretary's transfer authority?

Assisting Americans in gaining affordable health insurance is a top health care priority for the Department and will improve prevention efforts and public health. The new coverage options available in the Marketplace have increased access to preventive care and improved health outcomes for millions of Americans who have enrolled in affordable

private health plans or who plan to enroll in the next open season. Unfortunately, the HHS budget request for implementation of the Affordable Care Act was not fully funded in FY 2014. The Department thus leveraged its existing resources, including the transfer authority, to provide short term and immediate funding for these efforts.

1G) How much do you expect the full cost of implementation of ObamaCare from when it passed to complete implementation to cost the taxpayer?

Since enactment through February 28, 2014, the Department has obligated \$2.2 billion to implement and begin operations of the Marketplace. In addition \$2.0 billion in grant funding has been spent to states for their work to implement Marketplaces. Beginning in FY 2014, CMS is collecting user fees from issuers to fund ongoing operations costs and the final establishment grants will go to States at the end of 2014.

1H) What steps have you put in place to ensure there is full accountability for all the ObamaCare funds and how do you validate that these funds are spend appropriately?

Funding for Marketplaces is subject to the same types of financial controls that CMS employs for compliance with OMB Circular No. A-133 in its other programs. CMS is also subject to a separate audit as part of the HHS-wide Chief Financial Officer's audit. Additionally, in the FY 2015 President's Budget includes \$25 million of discretionary HCFA funding for program integrity activities in private insurance, including the Health Insurance Marketplace.

1I) Does the FY 2015 request for CMS Program Management assume:

- a) **Use of the Implementation Fund? If so, how much?**
- b) **Use of the Nonrecurring Expense Fund (NEF)? If so, how much?**
- c) **Use of the HHS 1 Percent Transfer Authority? If so, how much?**
- d) **Use of PHS Eval Set-Aside? If so, how much?**
- e) **Use of Prevention and Public Health Fund? If so, how much?**
- f) **Realignment of any FY 2014 CMS Program Management Funds being used for non-ObamaCare activities? If so, how much?**
- g) **Use of the special funds provided to CMS for traditional Medicare activities in the FY 2014 Appropriations Act General Provision? If so, how much?**
- h) **Use of any mandatory funds to support CMS Program Management activities related to ObamaCare? If so, how much?**

The \$629 million discretionary request for CMS Program Management combined with \$1.2 billion in Marketplace-related mandatory user fees would be sufficient to support Marketplace activities in 2015. HHS has used the 1% transfer authority and Nonrecurring

Expenses Fund in the past to finance necessary and discrete activities for implementation for which other funding was not made available. HHS expects the Implementation Fund to be exhausted by the end of FY 2014.

1J) Please explain if HHS expects Risk Corridor receipts are not as expected or at the anticipated outlay requirement rate, how will HHS deal with any expected shortfall? If it expects to use mandatory funds, please provide the legal opinion to justify such action? Can these requirements be shifted to another fiscal year? And finally, what is the impact of the program if these receipts are not at the anticipated requirement level?

HHS expects that the risk corridor program will be budget neutral over three years. The Congressional Budget Office (CBO) has consistently estimated since enactment in March 2010, and as recently as April 2014, that the program would be budget neutral or make a profit. This means that risk corridor collections from issuers are expected to fully offset payments to issuers. The CBO has never projected a deficit for this program.

HHS will implement the risk corridor program as a discretionary user fee program under the Centers for Medicare and Medicaid Services (CMS) longstanding user fee authority.

1K) What is the maximum amount of funds that could be available for the NEF in FY 2015? What is the FY 2015 budget request level expected level of funds available for FY 2014 and FY 2015?

The Department notified the Committees on Appropriations on October 18, 2014 for \$600 million in planned uses of the Nonrecurring Expenses Fund (NEF). As of March 31, 2014, approximately \$210 million had been obligated pursuant to the notification. HHS has transferred nearly \$300 million into the NEF this fiscal year. HHS expects to transfer and obligate the full amount of the notification, though exact timing is dependent on contracting procedures and subject to variability. The variability and unpredictable nature of eligible balances for the NEF comes from the requirements in 31 U.S.C. 1551 – 1558 to maintain expired unobligated balances in accounts for routine adjustments to previously recorded obligations. As an account nears its time of cancellation, HHS is able to identify amounts to transfer.

1L) Please describe the mechanisms in-place to ensure fiscal responsibility of the state exchange funds and the funds used to support the federal exchange? What is changing in FY 2014 and FY 2015 to increase oversight?

All funds for the establishment of State-based Marketplaces (SBMs) have been allocated through the standard grant-making process. CMS has overseen these grants using their existing grant management process, which reflects established policies and regulations and is designed to ensure that grants have continuous oversight. States must comply with extensive terms and conditions once a grant award is made. All states that receive grants under section 1311 of the Affordable Care Act must undergo establishment reviews, a critical component of the grant monitoring process. This establishment review process provides a benchmark not only for programmatic progress, but also financial accountability.

CMS will conduct diagnostic visits to the few states that have experienced technical challenges in identifying system defects and examine the management and decision-making process of the SBM in order to improve their future efficiency. CMS has put in place Corrective Action Plans for states that continue to have technical problems. In the states where SBMs remain on schedule and are functioning well, CMS will conduct site visits to monitor their continuing progress. For all SBMs, CMS will continue to provide both technical assistance and oversight in advance of the open enrollment period for the 2015 coverage year.

CMS funding for Marketplaces is subject to the same types of financial controls that CMS employs for compliance with OMB Circular No. A-133 in its other programs. CMS is also subject to a separate audit as part of the HHS-wide Chief Financial Officer's audit.

1M) Please describe the Transition Reinsurance Program, cost, and how the ObamaCare would be impacted if this particular activity did not exist? Please provide the same for Risk Adjustment Program and separately for the Risk Corridors activities.

The three premium stabilization programs were established by Congress in the law to mitigate volatility of insurance premiums in the individual and small group markets beginning in 2014 when Marketplaces and new market rules take effect. The transitional reinsurance program provides protection to plans that comply with the new market rules in the individual market when enrollees experience high claims costs for plan years 2014 through 2016. In FY 2015, CMS expects to make \$10 billion in reinsurance payments, which is funded through collections from issuers and group health plans. The temporary risk corridors program protects qualified health plans, as defined in 45 CFR 153.500, from uncertainty in rate setting from 2014 to 2016 through shared risk in issuer gains and losses. In FY 2015, the President's Budget assumes \$5.4 billion of risk corridors payments will be made, funded through \$5.4 billion in collections from plans that owe risk corridor payments. The permanent risk adjustment program transfers funds from plans with lower-risk enrollees to plans with higher-risk enrollees to protect against the

potential effects of adverse selection inside and outside the Marketplace in the individual and small group markets. CMS estimates \$3.4 billion will be paid in risk adjustment payments, which are funded through charges to plans with lower-risk enrollees.

1N) How much money are you requesting from this subcommittee in FY15 to support Obamacare Marketplaces? And that is an increase of exactly how much over FY14?

The FY 2015 Budget requests \$629 million in CMS Program Management for Marketplace operations. This is a decrease of approximately \$900 million below the \$1.5 billion requested in the FY2014 President's Budget due largely to increasing user fee collections.

1O) What other sources of funding does your budget assume will be used to support the Marketplaces in FY15? For example, do you plan to use funds from the Nonrecurring Expenses Fund in FY15 to support Obamacare? How much? Do you plan to use your 1% transfer authority to move money out of biomedical research and public health activities to support administration of Obamacare as you have the past two years?

In addition to the \$629 million request for discretionary Program Management resources, CMS will use \$1.2 Billion in user fees collected for CMS Marketplace costs.

1P) If user fees do not meet your estimates, as they did not last year due to the low numbers of enrollees, will you seek additional funding to administer the Marketplaces program from this Subcommittee?

The Marketplace began collecting user fees concurrently with the first month of coverage, which was January 2014. We had no expectation of collections in FY 2013. We note that our user fee estimates for FY 2014 were adjusted in part due to updated enrollment projections, but also to account for lower than projected premium rates in the individual and small group markets. We expect that with better data we will become increasingly more precise in our estimates of user fees and will fund the majority of Marketplace operational costs through this source.

1Q) What is the total cost to administer ObamaCare in FY 2015, from all sources?

The total CMS costs for Marketplaces in the FY 2015 President's Budget is \$1.8 billion, including \$1.2 billion in user fees and \$629 million from the CMS Program Management appropriation.

2. Federal Exchanges – BackOffice Functions:

At a House Energy and Commerce Committee hearing on November 19, 2013, Henry Chao, the CMS Deputy CIO who oversaw the technical development of the federal exchanges, said that his team had not yet completed 30 to 40 percent of the overall project.

Further, he noted that CMS was still working on a number of “back office” functions, to include the system to send payments to insurance companies, accounting system, and components to ensure the exchanges have accurate and matching information about enrollments. According to press reports, he stated that these systems must be in place by January.

Are the back office operations and IT support functions fully operational and secure?

- If not, when will all functions be fully operational and the security tested?
- If they are not operational or secure, how will this impact operations and costs?
- How many individual enrollees payments were processed through the exchanges back office functions in February 2014 and in March 2014?
- During the rollout debacle, it was revealed that HHS and the White House had received early warnings related to IT and security issues. Are there *any* other concerns that have been raised to you or your staff on any aspect of Obamacare to date?
- Your Administration has twice delayed a mandate that requires large employers to provide health care coverage or pay fines. I believe that companies with 51 to 100 employees now can wait until 2016 to cover their employees while those with over 100 employees will start these obligations in 2015. This month the Administration agreed to allow existing health care plans that would not otherwise be “qualified” to be offered to continue through 2016.
 - Does HHS plan to delay any other Obamacare deadlines, such as the SHOP exchanges, or any other aspect?
- In the hearing you stated that you would provide the legal opinion to support the delays made by the Administration. Please provide the

full legal opinion for each delay or change made as it related to the implementation of ObamaCare.

The automated payment and reporting system between issuers and CMS is not complete or fully tested. CMS has an interim process for paying issuers that are owed Marketplace financial assistance in the form of APTC or CSR payments. Under this interim process, issuers who are owed payments submit initial, aggregate information on a monthly basis in order to receive Marketplace financial assistance payments. This data includes preliminary total effectuated enrollments, enrollees receiving Marketplace financial assistance, and the estimated amount owed to the issuer, all of which are subject to change and unconfirmed by CMS. On a monthly basis, CMS compares the effectuated enrollment counts submitted by the issuers to the enrollment counts generated from the FFM for individual market medical issuers. These data and payments will be further reconciled once the automated payment and reporting system is in place.

3. CMS Center for Medicare and Medicaid Innovation (CMMI)

The Innovation Center was established in the Affordable Care Act to test “innovative payment and service delivery models to reduce program expenditures.”

My understanding is that the Innovation Center’s mandate is essentially to fund grants. It was created in ObamaCare with \$10 billion that is available only for the CMMI specific purposes through fiscal year 2019.

I was surprised to see on page 329 of the CMS budget document, that number of full-time employee equivalents (FTE’s) for this program has increased from 68 FTE in 2011 to almost 450 FTE in FY 2015 – that is an increase of more than six times the 2011 level.

Is this accurate?

What are they doing that requires so much staff?

How many staff are needed to fund grants?

Do they have contractors as well?

Are any of these employees involved in other Obamacare implementation activity?

Do any of them work on the Marketplaces?

It seems odd for a grant making program to need almost 450 FTEs. If this is the case, it seem like any savings they might find will be

absorbed by their own administrative costs. Please justify the FTE and contract support level of this program.

The Center for Medicare and Medicaid Innovation (Innovation Center) was created by section 3021 of the Affordable Care Act for the purpose of testing “innovative payment and service delivery models to reduce program expenditures ...while preserving or enhancing the quality of care” for those individuals who receive Medicare, Medicaid, or Children’s Health Insurance Program (CHIP) benefits. The Innovation Center designs, and develops projects to test new payment and service delivery models, evaluate results, advance best practices, and actively engage a broad range of stakeholders in this process. Only a small subset of the Innovation Center models, such as the Health Care Innovation Awards, take the form of competitive grant programs. The majority of the models are payment and delivery system models for Medicare, Medicaid, and CHIP beneficiaries that test broader changes and improvements in these programs. These models involve agreements with the model participants.

The Innovation Center is currently testing nineteen models. These models include accountable care organizations, primary care transformation initiatives, bundled payments for episodes of care for the treatment of certain health conditions, Health Care Innovation Awards, State Innovation Models, initiatives focused on Medicaid beneficiaries, and initiatives focused on Medicare-Medicaid enrollees. The Innovation Center solicits and selects organizations to participate in model tests through open, competitive processes. The process follows established protocols to ensure that it is fair and transparent, provides opportunities for potential partners to ask questions regarding the Innovation Center’s expectations, and relies on multi-stakeholder input to inform selection of the most qualified partners.

The management of the model, including the enrolling of provider participants in the model, sharing of data, ongoing monitoring of the model, and the shared learning systems, among other functions, requires ongoing interaction with the participants of the models, substantially more than is typically required with grants. The Innovation Center uses contractors to help develop, implement, and evaluate the models and overseeing the contractors is an additional responsibility that requires federal staff. Many of the payment models require changes to existing Medicare and Medicaid payment processes and payment systems and the specification of those changes is done collaboratively between staff in the Innovation Center and other parts of CMS, such as the Center for Medicare and the Center for Medicaid and CHIP Services. The findings will inform possible changes in health care payment and policy, as well as the development and testing of new models when necessary or appropriate.

The Innovation Center provides frequent feedback to providers who participate in each model in order to support continuous quality improvement, with the understanding that learning and adaptation are essential to enable providers and health systems to achieve the greatest efficiencies and improvements possible in each new payment model. The Innovation Center leverages claims data to deliver actionable feedback to providers about

their performance, and encourages participating providers to use their own performance data to drive continuous improvement in their outcomes.

The Innovation Center conducts a rigorous evaluation of each new payment and service delivery model tested. Statute specifies that measures in each evaluation must include an analysis of the quality of care furnished under the model (including the measurement of patient-level outcomes and patient-centeredness criteria) as well as changes in spending. The Innovation Center intends to use the evaluation results of the model's impact on quality of care and spending to provide information to the Secretary to enable the Secretary to make well-informed decisions on the potential expansion, termination, or modification of a model.

Every test of a new service delivery or payment model developed by the Innovation Center also includes a plan of action to ensure that the lessons learned and best practices identified during the test can be spread as widely and effectively as possible to support improvement for both CMS and the health care system at large. Evaluation results are shared with participating providers on an ongoing basis in order to promote more rapid learning. The Innovation Center has also created communication and information sharing mechanisms for participating health care providers that support broad and rapid dissemination of evidence and best practices that have the potential to deliver higher quality and lower cost care for Medicare, Medicaid and CHIP beneficiaries.

The Marketplaces are administered and overseen by the Center for Consumer Information and Insurance Oversight (CCIIO). No FTEs assigned to CCIIO are paid with funds provided under section 3021 of the Affordable Care Act.

As noted on page 329 of the CMS 2015 Congressional Justification, in FY 2011 (the first full year after the Innovation Center was created) 68 CMS FTEs were funded under section 3021 of the Affordable Care Act, and CMS estimates that number will be approximately 443 in FY 2015.

4. PHS Evaluation Set-Aside (a.k.a. TAP)

HHS is authorized to tax or as HHS refers to it, "tap" PHS Act authorized programs up to 1% of their appropriation in order to conduct program evaluations. The Administration has yet again requested language to increase the "tap". The 2015 request proposes to take the "tap" to 3 percent or effectively move around more than \$1.2 billion of resources through this non-transparent budget gimmick.

I am not sure why we need to continue the use of this mechanism that reduces public transparency. For example, while the request purports to provide NIH with \$30 billion for biomedical research, in reality, the PHS Set-Aside TAP reduces the NIH funding level by an estimated \$850 to \$900 million – These resources are then shifted to other activities within HHS.

The intent of this authority is to provide support for program evaluations. In reality, the funds generally support program operations within the Office of the Secretary, CDC, AHRQ, and other HHS agencies.

I would like to ask for your commitment to work with me to eliminate the use of TAP?

Can you commit to submitting a budget in FY 2016 that does not include the use of the TAP and reflects the actual resources each HHS agency, program, and activity requires?

The Public Health Service (PHS) Evaluation Set-Aside is authorized by section 241 of the PHS Act, which has been amended in appropriations bills, to fund activities across HHS like AHRQ and CDC's National Center for Health Statistics. These funds are used to support critical public health and evaluation activities across HHS. Ultimately Congress sets the percentage and usage of these funds through the appropriations process and specifies each amount in appropriations bill language. The FY 2015 President's Budget proposes an increase of the PHS Evaluation Set-Aside from 2.5% to 3% and transparently reports how this funding would be used, both in program level totals and in appropriations language.

5. Preventive Health and Health Services Block Grant (CDC)

I am a little confused. As a former Governor, I would assume that you would be a supporter of the Preventive Health and Health Services Block Grant (PHHSBG).

For over 30 years, the Prevention Block Grant has been a vital source of funding, allowing each state to address its most critical public health needs. Our understanding is that states use these flexible dollars to offset funding gaps in programs that address the leading causes of death and disability. The funds also enable states to respond to unanticipated or emerging public health threats.

Last year HHS proposed to eliminate this activity and the Omnibus restored these grants at an all-time high level funding level of \$160 million. Now again, the FY 2015 request proposes to eliminate the Preventive Health and Health Services Block Grant program.

Can you help us understand why HHS believes that this program that provides flexibility to States to address their most critical public health needs is not needed, especially at a time that CDC total program level is being reduced by \$243 million or almost 4 percent in FY 2015?

When the Preventive Health and Health Services Block Grant (PHHSBG) was first authorized in 1981, there were minimal resources within CDC's budget allocated for categorical programs such as heart disease, diabetes, immunizations, and obesity, and many states did not receive funding from CDC to support prevention of chronic disease. However, since 1981, categorical programs at CDC have grown to over \$1 billion annually and the PHHSBG now represents a much smaller percentage of state budgets when compared to total available CDC funding.

The FY 2015 budget request continues the elimination of the PHHSBG, which was also proposed for elimination in FY 2014. These activities may be more effectively and efficiently implemented through the State Public Health Actions to Prevent and Control Diabetes, Heart Disease, Obesity and Associated Risk Factors and Promote School Health program, which provides resources to states to coordinate activities across categorical funding streams, as well as Affordable Care Act Prevention and Public Health Fund investments. Elimination of this program provides an opportunity to find savings in a constrained budget environment, while enhancing functionality for core chronic diseases through other mechanisms.

6. Augmenting Discretionary Based Programs with Mandatory Funds

It appears the President's budget is using mandatory funds to drastically increase the funding base of existing programs and activities. In some instances the budget proposes to shift the program entirely over to mandatory funds, such as the request to transition the Children's Hospital Graduate Medical Education (GME) program over to a new \$5.2 billion mandatory funded graduate medical education program.

In other instances, the budget simply proposes to augment existing discretionary funded programs and activities with mandatory funds. For example, the budget proposes to expand the existing National Health Service Corps program. But the proposal only requests to drastically increase the mandatory funding by adding \$4 billion over 5 years and yet makes no similar request to increase the discretionary funding for the program.

Finally, the budget also proposes to simply extend Obamacare mandatory funds that were set to expire, such as the Community Health Center mandatory funds that were set to sunset after FY 2015.

- As an appropriator, I find it disturbing to see what are historically discretionary funded activities becoming exclusively or primarily mandatory funded programs. Moreover, this appears to be a back door approach to increasing the funding for programs.**

- **Can you please explain why there's such an emphasis on increasing mandatory funding of existing programs?**

The President's Budget renews our commitment to critical health care and workforce services now, before these essential programs expire, to continue the momentum built in the last five years.

As we look to full implementation of the Affordable Care Act this year and beyond, there is still work to be done to ensure that essential health services, like primary care and mental health, reach all Americans. These investments, which aim to improve access to health care and our health workforce, are challenging and take several years to realize results. It is important that we make a commitment to our communities, universities, and training partners that go beyond one year, and it is not uncommon to request mandatory funding for such investments.

Two areas where we have requested additional mandatory funding is for the Health Centers and the National Health Service Corps programs. Those who are now eligible for insurance through the Marketplaces or Medicaid expansion are historically low-income Americans who have relied on health centers for discounted care. These patients are likely to continue seeking care at health centers because of the high patient satisfaction rate, availability of comprehensive services, and established relationships with doctors and staff. Further, health centers will continue to serve patients who are medically underserved, as well as provide services that are not typically covered by insurance. The Budget's investment in health centers works in tandem with the National Health Service Corps proposal, as approximately 50 percent of Corps providers work in health centers around the country. In addition, investments in the Corps in FY 2015 will address health professional shortages in other high-need rural and urban communities across the country.

7. State Exchanges:

Section 1311 of the law says that each state "shall" establish an American Health Benefits Exchange by January 1, 2014. After spending an estimated \$261 million, Maryland established such an exchange. But it has been plagued by a number of problems. The result is an IG investigation into the situation there. If Maryland can no longer administer an exchange, is your department prepared to take over those responsibilities in Maryland and other states, such as Oregon or Hawaii or Minnesota, where there have been administrative or enrollment problems? What metrics is HHS using to decide to spend good money after bad on state exchanges?

CMS is providing help through technical assistance and financial assessments for states, including Maryland, that have had implementation challenges with their State-based Marketplaces. States have identified their functionality problems and implemented

mitigation strategies under CMS guidance. CMS has put in place Corrective Action Plans for states that continue to have technical problems. In the event that a State-based Marketplace is unable to continue operations, the Department is committed to ensuring that residents of such a state continue to have access to qualified health plans in the Marketplace.

8. ObamaCare Tax:

There is 3.5 percent administrative fee imposed on residents of states where the exchange is run by the federal government. Mr. Cohen told Congress in previous testimony that the percentage might have to be increased next year. Could you tell the Committee when you expect to finalize the payment parameters rule for 2015?

Please explain how these funds are being used and what criteria are used to set or adjust this tax rate?

We plan to finalize the payment notice by later this Spring.

OMB Circular No. A-25R establishes Federal policy regarding user fees, and specifies that a user charge will be assessed against each identifiable recipient of special benefits derived from Federal activities beyond those received by the general public. Circular No. A-25R states that user charges should generally be set at a level so that they are sufficient to recover the full cost to the Federal government of providing the service when the government is acting in its capacity as sovereign (as is the case when HHS operates a Marketplace). Consistent with OMB Circular No. A-25R, CMS charges user fees based on a special benefit being provided, i.e., cost to operate the Federally-facilitated Marketplace functions. CMS has also considered other factors, such as aligning with administrative cost structures of State-based Marketplaces.

Update: The Final HHS Notice of Benefit and Payment Parameters for 2015 was published in the Federal Register April 11, 2014.

9. Political Appointee's In ObamaCare:

According to April 12 2010 edition of *The New York Times*, President Obama agreed to volunteer to enroll in a health insurance exchange. For the record, are you enrolled in the health insurance exchange? How many Cabinet members are enrolled? Finally, how many political appointees are at HHS and of them, how many are enrolled in the health insurance exchange?

The Department of Health and Human Services does not have access to individual's personal health insurance information. The Department cannot provide this information.

10. Lake Lynn – Underground Mining (CDC)

In response to the Upper Big Branch explosion in April 2010, which killed 29 miners the CDC received a specific appropriation to ensure it maintained a physical underground mine facility. The CDC currently retains \$14 million in prior year appropriations for the Lake Lynn Laboratory.

In the March 15, 2013 response to questions for the record, CDC advised that it is moving forward to replace the mine safety research facility. In addition, CDC has advised Congress on numerous times that they are in the process to search for a replacement facility while some of this research is on hold. However, although it is not described in the HHS or CDC budget request, Congress has heard that CDC has made an arbitrary decision to abandon previous efforts to identify and select a replacement site, and the CDC intends to “provide a reprogramming notification to Congress to redirect these funds to high priority buildings and facilities repair and improvement projects.” Is this correct? Please explain how this action is consistent with what CDC has officially stated on the record to be moving forward to replace the mine safety research facility”?

Since the Lake Lynn limestone mine was developed in 1982 by the Bureau of Mines, it has served an essential role in simulating full scale gas and coal dust explosions and developing control technologies to prevent future mining disasters. Explosion tests conducted at Lake Lynn provided the data needed to update the MSHA regulations to prevent massive coal dust explosions, such as Upper Big Branch explosion in April 2010, which killed 29 miners. Lake Lynn mine was also used to develop standards for improved mine seals following the explosion in the Sago mine which took the lives of 12 miners on January 2006. Please explain how this is justifiable and why it is not proposed in the FY 2015 budget request as these funds are not available for reprogramming as they are specified for a specific purpose.

After being required to vacate the Lake Lynn site in 2012, CDC conducted a number of exploratory activities to replace the one-of-a-kind facilities at Lake Lynn – including awarding a contract to develop the criteria required for a replacement site and facilities. Although CDC has approximately \$14 million set aside for the replacement of Lake Lynn from funds appropriated by Congress in prior years, the cost to purchase and build a site is estimated at close to \$65 million. Because the \$14 million is insufficient to cover the full cost of acquiring and developing a new site, and it is not likely under current fiscal constraints, that CDC will obtain the funding necessary to fully replicate the

site, the Administration has decided not to continue to pursue a replacement site at this time.

The \$14 million that CDC currently has set aside for Lake Lynn was originally appropriated in FY 2009 to support acquisitions and construction projects on CDC campuses in Atlanta and other parts of the country, which are now complete. The FY 2011 Senate Report included support for Lake Lynn repair, but the enacted appropriations bill and related Report did not.

Mine safety research remains an occupational research priority for CDC. NIOSH will continue to carry out research projects in the areas of mine explosion, dust exposure and fire suppression as far as they can be taken in a laboratory setting, but the full complement of this work will not be achieved until an alternative for testing of NIOSH laboratory findings can be identified.

11. Prescription Drug Overdose (CDC)

The CDC FY 2015 budget request describes a proposal for injury prevention core support by \$15.6 million in FY 2015. In part these funds can be used to support Prescription Drug Overdose. Please identify how much of the FY 2014 funds in this program specifically support prescription drug overdose reduction activities? How are they measured? How are these activities selected? Plus, how much of the increase can you ensure the Committee will specifically support prescription drug overdose, how will it be tracked, and are these states support the locations with the most deaths due to drug overdose. In this program, how will or how does CDC ensure these funds support best practices and coordination with other federal and local programs.

Sixteen out of the twenty states currently funded Core Violence and Injury Prevention Program (Core VIPP) have prescription drug overdose (PDO) as one of their injury prevention priorities. As part of their base funding, states are awarded \$250,000 for their injury prevention activities, including PDO, but CDC does not currently track the percentage of funding used for each topical activity.

During the first year of funding, in addition to identifying focus areas to address during the five-year funding period, Core VIPP states are required to implement activities to build a solid foundation for violence and injury prevention activities; develop health impact measures for the focus areas; collect baseline data for the health impact measures; identify evidence-based program and policy strategies to address the focus areas selected; and develop and implement plans to evaluate the strategies. Such requirements help to ensure the use of Core VIPP funds to address pressing injury prevention priorities, establish a strategic means for measuring and tracking progress, and maximize existing

infrastructure in partnership with other entities within the state and existing related programs.

For the proposed additional funding outlined in the FY 2015 President's Budget, CDC estimates approximately 75% of the funding will go out to support PDO work at the state level, in two ways: through Core VIPP's Base Integration Component and through additional funding and scientific assistance. CDC will provide base injury prevention funding to a number of states that are not currently part of the Core VIPP program, with an emphasis on states with the highest burden of PDO. The goal is to build a state's basic ability for injury prevention in order to have a foundation for PDO-specific activities. Each of these states will be required to include PDO as one of their injury prevention priorities and align CDC's PDO strategies. The majority of the funding will be used for a set of Core VIPP states to expand and intensify their PDO prevention activities. This funding will be competed among existing and newly funded Core VIPP states, with an emphasis on states with the highest burden of PDO and those states most ready to conduct PDO prevention activities.

The additional funding for PDO prevention activities will focus on implementation of specific interventions aligned with the following priority areas for investment: assisting insurers and clinicians in improving coordination of care for patients at high-risk of overdose (i.e. evaluating insurance innovations); supporting development and effective use of prescription drug monitoring programs (PDMPs); and evaluating programs and policies to build the evidence base for overdose prevention. These activities are complementary to and help to inform efforts of other federal agencies, including substance abuse prevention and treatment efforts at SAMHSA and law enforcement efforts at the federal, state, and local levels.

12. Advanced Molecular Detection (CDC)

Please describe how the AMD program supported in FY 2014 is aligned to the FY 2015 requested for additional advance development activity? Please provide the total costs for all this similar CDC activity in FY 2015 and prioritize these actions in order of highest priority for each specific activity. Further, please show where the base funds provided in FY 2014 are in the FY 2015 request?

The FY 2015 President's Budget requests continued investment of \$30 million for CDC's AMD activities. AMD activities proposed in FY 2015 will align to those started in FY 2014. FY 2015 AMD activities will continue to expand the use of high throughput sequencing and bioinformatics approaches for responding to major outbreaks, expand the use of molecular sequencing for surveillance and outbreak investigations, and work to establish highly curated reference databases for pathogen identification. The dataset generated from CDC's initial efforts on analysis of reference and outbreak samples will speed the development of updated and more precise pathogen detection and strain identification techniques. Requested FY 2015 AMD funding will be used to advance prevention of infectious diseases across numerous programs at CDC, including foodborne

illnesses, influenza, hepatitis and arboviruses, among others. Within the FY 2015 request, AMD priority areas and activities are identified.

FY 2014 budget authority for AMD was appropriated to CDC's Emerging and Zoonotic Infectious Diseases account. The FY 2015 President's Budget requests continuation of that funding.

13. Obesity Outreach (CDC)

Please explain why CDC decided not to support the FY 2014 obesity outreach program? How was it evaluated?

CDC is supporting the obesity outreach program through the Childhood Obesity Research demonstration Project (CORD) which awarded funding to 3 demonstration sites (Texas, Massachusetts, and California) and an evaluation center in Houston, Texas in September 2011. The funded sites and evaluation center were forward funded for the entire five year project period. The CORD project targets underserved children ages 2 to 12 years of age. The focus of the project is to improve risk factors for obesity for the underserved children. Funded strategies include: improved physical activity, sleep, fruit and vegetable intake, reduced television and screen time viewing, high energy dense foods and sugar drink consumption, improved utilization of preventive services such as screening and counseling, improved satisfaction with health care services, and improved quality of life.

The CORD project was derived from an inter-agency agreement with CMS and forward funded for the full five year period. Grantees are still in the implementation phase. Some early success stories are currently under development. Evaluation of this research project, including project outcomes, will be ready January 2016. This demonstration project will end after five years, and will use the lessons learned to inform CDC's childhood obesity efforts overall.

14. Global Health Strategy plan

Please provide an update on actions planned in FY 2014 and 2015 related to the Global Health Strategy plan with CDC, FDA, and NIH as described in the FY 2014 statement of manages.

Please explain how the activity described in the FY 2015 budget request for CDC and NIH fit into measurable objectives to this plan and explain how each was coordinated.

The Consolidated Appropriations Act of 2014 requested that CDC's Global Health program lead the preparation of a CDC, FDA, and NIH joint plan for a global health strategy. Such a strategy already exists within the Department. In 2010, the Department developed a Global Health Strategy, covering the period 2011 to 2015. This strategy was developed under the leadership of the Office of Global Affairs within the Office of the

Secretary, working in close coordination with key operating divisions, including CDC, FDA, and NIH. The HHS Global Health Strategy includes ten key objectives: (1) enhance global health surveillance; (2) prevent infectious diseases and other health threats; (3) Prepare for and respond to public health emergencies; (4) increase the safety and integrity of global manufacturing and supply chains; (5) strengthen international standards through multilateral engagement; (6) catalyze health research globally; (7) identify and exchange best practices to strengthen health systems; (8) address the changing global patterns of death, illness and disability; (9) support the Global Health Initiative; and (10) advance health diplomacy. The Department is currently drafting a formal response to the request for a global health strategy plan in the Statement of Managers.

The Department and its operating divisions, including CDC, FDA, and NIH, work in a coordinated manner to protect and promote the health of the American people. For instance, CDC, NIH, and FDA are collaborating with other USG and foundation partners to support a polio antivirals initiative. Led by the Task Force for Global Health, a non-governmental organization, the polio antivirals initiative aims to facilitate rapid development of at least two safe and effective polio antiviral drugs. In addition, CDC, NIH, FDA, and the Biomedical Advanced Research and Development Authority (BARDA) work together to improve pandemic vaccine technology and manufacturing, as well as with NIH-funded researchers to monitor the evolution of animal influenza viruses with pandemic potential and leads in conducting risk assessments of these viruses and developing candidate vaccine strains when appropriate. Furthermore, FDA, NIH, and CDC are collaborating to increase the safety and integrity of global medicine supply chains by testing tools, such as the Counterfeit Detection Device to identify counterfeit medicines.

CDC's global activities described in the FY 2015 President's Budget are linked to the agency's own Global Health Strategy, as well as to the objectives of the HHS Global Health Strategy. Some examples of linkages to the HHS Global Health Strategy objectives include:

- As the public health leader in preventing and controlling diseases, CDC plays an essential role in implementing the U.S. President's Emergency Plan for AIDS Relief (PEPFAR). CDC uses its technical expertise in public health science and long-standing partnerships with ministries of health (MOH) and global partners to build strong national programs and sustainable public health systems that meet the needs of each country, allowing an effective response to the HIV/AIDS epidemic. CDC provides HIV/AIDS scientific and programmatic support and mentoring through its headquarters in Atlanta and its 45 offices in Africa, Asia, Central America, South America, and the Caribbean. CDC's global AIDS program strengthens global health surveillance (objective 1) by building and maintaining public health systems needed to track the epidemic and inform prevention strategies and CDC's work in this area catalyzes research (objective

6), particularly research related to HIV prevention. Moreover, as an implementer of PEPFAR, CDC supports a key U.S Government objective of advancing global health diplomacy (objective 10).

- Through the Global Immunization Program, CDC protects the health of Americans and global citizens by extending the reach of proven, life-saving immunization practices. As a key implementer in the global partnership to eradicate polio, CDC's immunization program saves millions of lives and holds promise for ending the scourge of polio. Through the global immunization program, CDC provides epidemiological and laboratory surveillance expertise (objective 1); immunization campaign planning, implementation, monitoring, and evaluation as well as immunization system strengthening work (objective 7); and outbreak preparedness and response (objective 3).
- CDC supports prevention, control, elimination, diagnosis, and treatment of a wide range of parasitic diseases that threaten the health of individuals in the United States and globally. Through this work, CDC advances health diplomacy (objective 10) as a key implementing partner for the President's Malaria Initiative, through which CDC and partners work to lessen the impact of this high burden disease (objectives 2 and 8).
- Through the Global Disease Detection and Global Public Health Capacity Development Programs, CDC provides epidemic intelligence and response capacity for early warning about international disease threats and develops country-level capacities to ensure emergency preparedness and response to incidents of local and international importance (objectives 3 and 8). Building the capacity for a country to detect and respond to a potential disease outbreak or public health emergency before an event occurs helps contain dangerous pathogens as they emerge, thereby saving lives, protecting the global and U.S. economies, and preventing the spread of disease across borders (Objectives 1 and 2). CDC works with partners to build strong, nimble, and sustained public health systems by focusing on the foundational capacities of applied epidemiology, surveillance, policy development, informatics and health information systems, evaluation, research, and laboratory systems (Objective 7). Programmatically, CDC also ensures global health protection by implementing the International Health Regulations (Objectives 5 and 7).

NIH helps advance the Global Health Strategy by working in partnership with other international research funding agencies to lead a coordinated scientific agenda and approach for bringing science and new technologies to bear on health problems

throughout the world. Key NIH activities focus most closely on the following linked objectives of the Global Health Strategy:

- *Objective 2: Prevent Infectious Diseases and Other Health Threats*
- *Objective 6: Catalyze Health Research Globally*
- *Objective 7: Identify and Exchange Best Practices to Strengthen Health Systems*
- *Objective 8: Address the Changing Global Patterns of Death, Illness and Disability*
- *Objective 10: Advance Health Diplomacy*

The following are examples of NIH activities that advance the measurable objectives of the HHS Global Health Strategy.

HIV Prevention Science. For decades, the notion of controlling or ending the HIV/AIDS pandemic has been a distant aspiration. Because of gains in treatment prevention strategies made possible through research advancement, it has become possible for the first time in more than 30 years to anticipate an end to the pandemic. In FY 2015, multiple NIH components will work in partnership with Office of the Global AIDS Coordinator and CDC to support implementation science and specific projects to address such topics as Prevention of Mother to Child Transmission, integration of primary health care and HIV services, scale-up of male circumcision, and social/behavioral approaches to prevent transmission. The ultimate prevention tool is a safe and effective vaccine. Working in cooperation with partners, NIH has advanced research to isolate highly potent, broadly neutralizing antibodies from chronically infected patients. In order to help design and optimize preventive vaccines, NIH will work to increase the understanding of how these antibodies develop and mature in infected individuals.

Non-Communicable Diseases. Non-communicable diseases now account for 63 percent of deaths worldwide and is expected to grow, principally due to heart disease, diabetes, cancer, and chronic respiratory diseases. NIH research and scientific partnerships between U.S. and foreign research institutions will work to dampen this silent epidemic.

In the field of global cancer research, the National Cancer Institute (NCI) launched partnerships to foster new research projects, training, advanced technologies, and clinical trial networks that will help improve cancer research capacity in low- and middle-income countries. NCI also has created an initiative to support the development of new user-friendly, lower-cost devices or diagnostics that are clinically comparable to technologies in current use for cancer imaging, in vitro detection/diagnosis, or treatment of cancers.

A meaningful share of the global burden of disease is attributable to mental, neurological, and substance use disorders such as depression, alcohol/drug use, and suicide. In response, the National Institute of Mental Health will continue its investment in Collaborative Hubs for International Research on Mental Health, to increase the research base for mental health interventions in low- and middle-income countries. The program goals are to provide the necessary knowledge, tools, and sustainable research-based strategies for use by government agencies, non-governmental organizations, and health

care institutions in order to address urgent mental health diagnosis and treatment needs. Lessons learned from these contexts can inform mental health service delivery in low-resource settings in the United States and elsewhere.

Mobile Health Technologies. The enormous potential for mobile technology to transform diagnosis, monitoring and treatment of disease, health care delivery and personal health management, has led to the rapid development of new health-related phone applications. However, evaluation of health outcomes resulting from these applications is often lacking. A new NIH collaboration between six Institutes and Centers (ICs)—*Mobile Health: Technology and Outcomes in Low and Middle Income Countries*—will support the development of an evidence base for the use of mobile technology to improve clinical outcomes and public health, while also building research capacity in this emerging field and research networks in this field. This opportunity emphasizes tools or interventions for chronic diseases and technologies for cross-cutting applications. Several key mHealth tools are being tested in the field. For example, NIH-supported researchers are evaluating a quarter-sized, lens-less microscope that, when connected to a mobile phone, can beam high-quality images of cells and microbes halfway around the globe to computers that can automatically interpret the images. An even more affordable option under development is a paper microscope that costs about 50 cents to produce and requires no power supply.

Investments in Human Capital. Many countries lack sufficient numbers of trained scientists and researchers. NIH's Common Fund Program joined with the U.S. President's Emergency Plan for AIDS Relief (PEPFAR) and the Health Resources Services Administration to launch the Medical Education Partnership Initiative (MEPI) to help institutions in Sub-Saharan Africa develop or expand their health workforce and enhance models of medical education, as well as build clinical and research capacity. In addition, the Fogarty Global Health Program for Fellows and Scholars will support more than 400 early-career health scientists with focused mentoring and nearly year-long global health clinical research experiences at approximately 80 established research sites in 27 LMICs. The program will enhance the career trajectory of participants, strengthen global health research programs at U.S. and foreign institutions, and bolster collaborative networks among fellows, scholars, and senior mentors.

Partnerships in Small Molecule Research and Genomics. Through innovative public private partnerships, NIH and other government agencies have been involved directly in the development of 26 (59%) of the 44 products introduced to address neglected diseases in the past decade. A notable example of NIH's continuing commitment to strengthening the pipeline of drugs for rare and neglected diseases is the Therapeutics for Rare and Neglected Diseases (TRND) program. TRND is a congressionally mandated initiative to speed development of new drugs for rare and neglected diseases through milestone-driven drug development projects. Current projects of relevance for global health include the development of new therapeutic agents for schistosomiasis, sickle cell disease, and cryptococcal meningitis (the second leading cause of HIV-related deaths in the African sub-continent). In addition, the Human Heredity and Health in Africa (H3Africa) Initiative, a collaborative effort with the Wellcome Trust in the UK, aims to facilitate a

contemporary research approach to the study of genetic and environmental determinants of common diseases, with the goal of improving the health of African populations. The H3 Africa project involves establishing and applying cutting-edge genomic research tools and bioinformatics technologies in order to perform state-of-the-art biomedical research in the region. It may yield important leads in the development of future drugs and vaccines.

15. CDC Budget Information

The FY 2014 Statement of Managers required CDC to link each program to specific measurable public health and preparedness goals and objectives in the FY 2015 budget request. Due to timing, this action was not completed for FY 2015. Please provide a crosswalk of these links with measurable objectives and goals for each CDC program line in the FY 2015 budget request as compared to the FY 2013 baseline and FY 2014 projected performance. Further, please explain how CDC expects to incorporate this into the FY 2016 budget request.

CDC's 2015 Congressional Justification includes information about CDC's overarching goals and how investments address these goals:

- Protect Americans from infectious diseases
- Prevent the leading causes of disease, disability, and death
- Protect Americans from natural and bioterrorism threats
- Ensure global disease protection
- Keep Americans safe from environmental and work-related hazards
- Monitor health and ensure laboratory excellence.

Specific long-term objectives, measures, baselines, and targets and results for each of CDC's programs can be found in the Performance Section of the CJ, pp 336-416.

16. Scientific Research Coordination between CDC and NIH

Please describe what activities are assumed in the FY 2015 budget request to support increased coordination between NIH Institutes and Centers and the CDC programs and Centers to identify scientific gaps and accelerate understanding of diseases and their prevention knowledge. How will these activities be measured?

Both CDC and NIH are public health science agencies working to help people live longer and healthier lives, and the agencies collaborate on many activities. Though the skills and tools CDC and NIH deploy have commonalities – for example, they each maintain laboratories that are among the world's most advanced – each has a unique and complementary focus that contributes to our nation's health goals. While NIH conducts and funds basic and applied biomedical and behavioral research, CDC engages in health

promotion, prevention of disease, injury, and disability, and preparedness for new health threats. Given the complementary missions of CDC and NIH, the two agencies often work closely together to build on each other's respective strengths and achieve shared objectives. CDC and NIH partner on a diverse range of activities, such as co-funding research initiatives, co-developing health surveys, co-sponsoring scientific meetings and workshops, and engaging in interagency planning activities conducted through numerous committees and working groups.

Examples of ongoing collaborative activities include:

- CDC and NIH maintain a strong and active presence within the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) by participating in multiple teams and committees to ensure that the results of NIH-supported research can be translated rapidly into safe and effective medical countermeasures to emerging and reemerging infectious disease threats and to ensure coordination of scientific activity with PHEMCE partners. NIH research (along with that of the Biomedical Advanced Research and Development Authority BARDA and the DOD) contributes to the development of medical countermeasures to bioterrorist attacks. CDC works with communities to be prepared to manage natural or man-made emergencies and also provides disaster response assistance when requested. For example, the National Institute on Allergies and Infectious Disease (NIAID) collaborated with the CDC, FDA, and BARDA to rapidly develop and test a vaccine for 2009 H1N1 pandemic influenza, including in special populations such as the elderly and children. CDC also maintains the nation's Strategic National Stockpile, with the capability of deploying countermeasures to communities across the U.S. within hours in an emergency.
- Administered by NIH, the Disaster Research Response (DR2) Project coordinates efforts of CDC, the Agency for Toxic Substances and Disease Registry, FDA, and the HHS Office of the Assistant Secretary for Preparedness and Response to create a disaster research system consisting of coordinated environmental health disaster research data collection tools and a network of trained research responders. This effort will provide invaluable lessons and platforms for advancing timely post-disaster research activities.
- CDC and NIH worked together on clinical trials on diabetes prevention. NIH, CDC, and the Indian Health Service, support implementation of the National Diabetes Prevention Program in communities across the U.S. This long-term outcomes study has shown that diet and exercise or the diabetes medication, metformin, can delay the onset of diabetes by 10 years.
- NIH research helped develop drugs that control blood pressure and cholesterol; CDC works with clinicians, health systems, and the public health community to get people screened and treated -- with the goal of preventing a million heart attacks and strokes in 5 years.
- NIH research has identified the increasing range of diseases associated with tobacco use; CDC uses that research to work across the US to educate smokers, encourage them to quit, and support them with quitting resources.

- Research conducted by NIH and NIH grantees has helped further the development of many vaccines. As a public health agency working directly with the States, CDC helps to further efforts on the ground to ensure high public vaccination rates.
- To advance and accelerate progress in addressing the nation's childhood obesity epidemic, NIH and CDC, along with USDA and the Robert Wood Johnson Foundation, formed the National Collaborative on Childhood Obesity Research (NCCOR). Capitalizing on each other's strengths, the NCCOR partners aim to improve the efficiency, effectiveness, and application of childhood obesity research. Specifically, NCCOR seeks to:
 - increase surveillance of childhood obesity,
 - identify, design, and evaluate practical and sustainable interventions, and
 - support coordination and collaboration to halt and reverse childhood obesity.
- Using a database created by NIH to publish sequences of 100,000 bacteria known to cause foodborne outbreaks, NIH, CDC, and FDA are piloting a project to operate a pipeline for rapid pathogen identification of the bacteria involved in specific outbreaks and facilitate development of tests to aid diagnosis and treatment of affected people.
- The National Health Interview Survey (NHIS) (coordinated by the National Center for Health Statistics (NCHS) within CDC) has collected data on the nation's health since 1957 through personal household interviews. In collaboration with CDC, several of the NIH Institutes sponsor special topical supplements or help design questions for the survey. NHIS results provide data to better track health status, health care access, and progress toward achieving national health objectives.
- In a similar collaborative effort, NIH also partially supports the CDC's National Health and Nutrition Examination Survey (NHANES). NHANES is a large program of studies designed to assess the health and nutritional status of adults and children in the U.S.
- The Dietary Supplement Label Database is a searchable database of information taken from the labels of more than 20,000 dietary supplement products. NIH collaborates with CDC, USDA, FDA, and DoD to update the database monthly, providing important health information to the public.
- NIH and CDC also partner on surveillance efforts related to sudden death in the young (SDY) and recently launched a new registry to track cases of SDY (up to age 24), including sudden cardiac death and sudden unexpected death in epilepsy (SUDEP).
- Research conducted by NIH and others led to anti-retroviral drugs for HIV; CDC promotes testing so Americans know their HIV status and can seek treatment. Through PEPFAR, CDC has found cost-effective ways to treat millions of people worldwide. Sponsored by CDC, HRSA, and NIH's Office of AIDS Research, NIAID, and National Library of Medicine, AIDSinfo is a Web resource that offers access to the latest, federally approved HIV/AIDS medical practice guidelines, HIV treatment and prevention clinical trials, and other research information for health care providers, researchers, people affected by HIV/AIDS, and the general public

Within the FY 2015 President's Budget request, the below activities are examples of collaborative work between CDC and NIH that will continue to promote scientific research coordination:

- **Listeria Genomic Sequencing:** The Listeria initiative is an ongoing collaborative effort between CDC, NIH, FDA, USDA and several state partners to sequence and analyze clinical and environmental *Listeria monocytogenes* isolates in near real-time.
 - Participating laboratories at CDC and FDA upload sequence data to the Sequence Read Archive (SRA) at the National Center for Biotechnology Information (NCBI/NIH) within approximately one week of receipt.
 - The NIH provides a centralized database for these data, and provides weekly/on-demand analysis and feedback to CDC to facilitate the identification of outbreaks of listeriosis, the disease caused by *Listeria*.
 - CDC is working with NIH to optimize the uploading and analysis processes to further enhance the identification and investigation of outbreaks of foodborne infections, and with federal and state partners to develop a framework for data and metadata sharing and integration.
 - Since September of 2013, CDC has sequenced more than 450 samples of *Listeria* and sent them to NIH for analysis.
 - CDC Activity Measurement for this Collaboration: Through this large collaborative, CDC will continue to track the number of sequences that are done (450 to-date).

- **Valley Fever:** Randomized Clinical Trial, NIH lead. The goal of the trial is to assess the effectiveness of the antifungal drug fluconazole to treat Valley Fever in the context of community acquired pneumonia (CAP). The trial will enroll approximately 2,000 patients (1,000 in/near Bakersfield, CA and Phoenix/Tucson, AZ) diagnosed with community acquired pneumonia. CDC has provided the names of providers in Arizona (Tucson and Phoenix) and California (Bakersfield) who would be the first providers to see a patient suffering from CAP, and also information on the percentage of these patients who go on to develop Valley fever as a cause of CAP. NIH anticipates that the trial will begin in mid to late 2015.

CDC is supporting NIH's clinical trial by planning to conduct a large analysis of Valley Fever and community-acquired pneumonia cases in California:

- To identify demographic and clinical factors that are associated with being tested for Valley Fever, testing positive for Valley Fever, and experiencing poor outcomes.
 - This study will help inform the design of the NIH's trial, but also yield important information for local health departments and healthcare providers.
 - CDC's analysis for the study will help determine the number of enrollees for this trial.
- **Antibiotic Resistance:** CDC is collaborating with several federal agencies in the area of antibiotic resistance, and is continually looking for ways to make the data

it collects through surveillance efforts available to other agencies and public health authorities to maximize the U.S. efforts to combat antibiotic resistance. The Interagency Task Force on Antibiotic Resistance (ITFAR) provides specific information on what agencies are collaborating on in this area. Some highlights of ongoing collaborations include:

- CDC, FDA, NIH, DoD collaborating on laboratory data of antibiotic resistance threats. Multiple federal agencies are discussing how best to share lab and genomic data on antibiotic resistance threats across the government to support surveillance, research, and diagnostic development.
- FDA, NIH, CDC and other stakeholders will develop a publicly available diagnostic-level genome sequence database of bacterial strains. These will be deposited in NIH databases and a report will be developed and made available to the federal government, stakeholders and public. Among other applications, these data will be used to develop new antibiotic resistance diagnostics for more rapid and accurate detection of antibiotic resistance.
- NIH, with NIAID as the lead, collaborates with CDC and other Federal partners to address the important issue of antimicrobial resistance. NIAID co-chairs, along with CDC and FDA, the Federal Interagency Task Force on Antimicrobial Resistance (ITFAR). Recently, NIAID collaborated with CDC to conduct a clinical trial to identify new treatment options for gonorrhea, which is increasingly becoming antibiotic resistant. The results of the trial were used by CDC to update new treatment recommendations.

17. NIH Intramural Advance Coordination with Extramural
Please describe the mechanism NIH is using to provide advance consultation with the extramural community for intramural increases greater than fixed cost increases for pay raises.

The intramural budget has not had significant increases above those needed to cover fixed costs for pay raises. If an urgent need for additional investments in intramural research activities were to arise (such as required by a public health emergency or extraordinary research opportunity), it would be reviewed by the National Advisory Councils of the individual ICs and/or the Advisory Committee to the Director, or addressed through advice from the Director's Scientific Management Review Board, all of which include representatives from the extramural community.

18. NIH Administrative Burden Reduction Workgroup:
Please describe the mechanism and timeline NIH has established to establish the requested Administrative Burden Reduction Workgroup. Further, how is NIH reaching out to the community for participation and input?

NIH is committed to implementing processes for administrative oversight that minimize the burden for grantees and is involved in a number of activities to accomplish this objective.

NIH will continue to participate fully in opportunities to partner with universities, not-for-profits, and research institutes that receive federal grant support to develop methods to track and measure the administrative burden on entities. The Federal Demonstration Partnership (FDP) is an association of federal agencies, academic research institutions with administrative, faculty and technical representation, and research policy organizations that work to streamline the administration of federally sponsored research. The FDP sponsored a Faculty Burden Survey in 2007 and again in 2012 to assess the nature and magnitude of administrative burden among its member organizations. NIH leadership will extend an invitation to the FDP to partner with them to identify ways to reduce burden using the results of their recent survey, and to plan future assessments for ongoing tracking and measurement of administrative burden.

The Research Business Models (RBM) Working Group is an interagency working group of the Subcommittee on Social, Behavioral and Economic Sciences (SBE) of the Committee on Science (CoS) a chartered committee of the National Science and Technology Council (NSTC). RBM Working Group objectives include streamlining business models for the conduct of scientific research sponsored by the Federal government. Among the accomplishments of the RBM working group has been the development of a standardized uniform format for reporting performance on Federally-funded research projects. Development of standard reporting categories will facilitate the development of a common electronic solution for collecting information in lieu of collecting it through numerous agency-unique reporting forms currently used. The new Research performance Progress Report has directly benefitted award recipients by making it easier for recipients to administer Federal grant programs through standardization of the types of research information required in performance reports.

Finally, the NIH Scientific Management Review Board (SMRB), established pursuant to Sec. 401(e) of the PHS Act, is a statutorily-mandated committee to advise the NIH Director on the effective alignment of the Agency structure and function to achieve its goals through thorough evaluation and cautious consideration. At its May 7, 2014 meeting, the SMRB was charged with recommending ways to further optimize the process for review and award of NIH grants in a way that maximizes the time researchers can devote to research while still maintaining proper oversight. The SMRB will present its recommended strategies to reduce burden on recipient institutions and other parties involved in the grant-making process (i.e., scientific reviewers, Council members, and NIH staff), as well as to streamline the grant-making process. It is anticipated that the SMRB will be complete its deliberations by spring 2015.

19.AHRQ Mission

Please provide specific justification on why AHRQ wants to changes its mission from its traditional core areas to that of supporting health reform justification. What authorization authority is needed to support these new mission areas?

The AHRQ's mission, as stated in the FY 2015 Congressional Justification, was reframed in an effort to better reflect AHRQ's role in helping to build the evidence to make health care safer, higher quality, more accessible, equitable, and affordable. AHRQ will continue its role in its traditional core areas such as patient safety, preventing healthcare associated infections, health services research, the dissemination of patient-centered outcomes research, and the Medical Expenditure Panel Survey, as well as questions of insurance coverage, healthcare value, and effectiveness. As these activities fall within AHRQ's existing authorizations, we are not seeking additional authorization authority at this time.

20. ACA vs non ACA

Please provide a breakout of the ACA and non-ACA costs in the FY 2015 request, FY 2014 estimated, FY 2013 and FY 2012 actuals for CMS Program Management. In addition, provide a table to display any ACA actual and estimated costs FY 2012, FY 2013, FY 2014 and FY 2015 for each HHS OpDiv.

Information on ACA and non-ACA activities funded under Program Management for the FY 2012, FY 2013, FY 2014 and FY 2015 estimates is available in the FY 2013, FY 2014, and FY 2015 CMS Congressional Justifications. Also, see attached which reflects estimates in each OpDiv's 2015 CJ.

21. Lobbying Update

Please provide an update on how each OpDiv and HHS Office is measuring the effectiveness of ensuring no federal funds are used to support lobbying activities prohibited by law.

The Department is committed to ensuring the proper use of federal funds and compliance with all applicable restrictions on lobbying, and has in place long-standing Department-wide guidance to grantees, which has always prohibited lobbying at both the federal and state level. The Department has upheld this prohibition for years.

In April 2012, HHS revised the Department-wide guidance to reflect differences between the lobbying restriction provision in the FY 2012 Appropriations act and the analogous provision in prior years' acts. This updated guidance was provided to grantees via an Action Transmittal, which clearly communicated each of the policy requirements of the FY 2012 Appropriations provisions. The Department takes these requirements very seriously and will promptly address any issues.

22. Allocation Criteria & Procedures

Please explain the resource allocation criteria and procedures used by the Secretary in making funding allocation decisions for FY 2014 and FY 2015 for each of the following: Health Reform Implementation Fund; Transfer Authority; Prevention and Public Health Fund; and Nonrecurring Expense Fund.

Health Insurance Reform Implementation Fund

HHS is authorized to use the Health Insurance Reform Implementation Fund to support ACA implementation efforts assigned to HHS or its federal partners within the Act. HHS allocates resources from this fund each year in consultation with its federal partners based on needs that arise in implementing the Act in a coordinated manner. HHS anticipates that this fund will be exhausted by the end of FY 2014.

Transfer Authority

The Secretary's transfer authority allows the Department to respond to emergency needs or unforeseen events which would otherwise adversely affect a program or agency. The transfer authority may also be used to carry out Departmental priorities. Transfers are not used to permanently alter base resources but, instead, are authorized on a one-time basis to cover current funding requirements. The Department ensures that use of the transfer authority is the only appropriate arrangement or remedy for the funding need.

Prevention and Public Health Fund

Funding decisions for the Prevention Fund were made using the same formulation process used to develop the annual federal budget and was decided in conjunction with other annual budget decisions. HHS works with public health, programmatic, and scientific experts in agencies across the department to identify effective and proven strategies that will improve health outcomes, promote prevention, and aim to reduce the cost of health care. HHS considered the FY 2014 appropriations and Prevention Fund allocation in making decisions for FY 2015.

Nonrecurring Expenses Fund

The Nonrecurring Expenses Fund (NEF) authority is restricted to capital acquisitions necessary for the operation of the Department, including information technology infrastructure. The Department identifies critical infrastructure needs and prioritizes investments across the Department.

23. Management Controls Oversight

Please provide an update of the actions taken over the past year by OpDiv to improve oversight of management controls to ensure federal funds support only the stated purposes authorized by law.

In accordance with the *Federal Managers' Financial Integrity Act (FMFIA)* of 1982 and OMB Circular A-123, *Management's Responsibility for Internal Control*, HHS and its

operating divisions annually assess management controls to achieve the objectives of and mitigate risks related to (1) effective and efficient operations, (2) reliable financial reporting, and (3) compliance with applicable laws and regulations.

To support the improvement of management control oversight, the ASFR/OF released updated guidance, *FY 2014 HHS FMFIA Guidelines*, this guidance addresses responsibilities for oversight and management controls.

Specifically, HHS guidance requires each OPDIV's management to establish management controls and conduct assessments designed to provide reasonable assurance that:

- (i) obligations and costs are in compliance with applicable law;
- (ii) funds, property, and other assets are safeguarded against waste, loss, unauthorized use, or misappropriation; and
- (iii) revenues and expenditures applicable to agency operations are properly recorded and accounted for to permit the preparation of accounts and reliable financial and statistical reports and to maintain accountability over the assets.

24. Secretary NIH Funding

The 2015 NIH request includes an allocation of additional resources in a disproportional manner related to strategic choices. Please provide the detailed criteria and resource allocation method used by the Secretary to ensure alignment with the NIH Institutes and Centers (IC) funding allocation to the HHS Strategic Plan. For each NIH initiative, please provide the specific strategic linkage and the specific short and long-term performance measure for each for FY 2014 through FY 2019.

The FY 2015 NIH Congressional Justification (CJ) exhibit entitled "Budget by HHS Strategic Goal" (page ES-29) meets a requirement to fully distribute the President's Budget (PB) request across the HHS strategic goals. The increase shown for HHS Strategic Goal 1 (Strengthen Health Care) did not reflect any change in resource allocation, just a long-standing methodology that has become unreliable due to changing circumstances. There was no intent to reduce NIH's organizational emphasis on Strategic Goal 2 (Advance Scientific Knowledge and Innovation) in FY 2015. Since FY 2006, the funding distribution in this exhibit has been developed by associating NIH's Government Performance and Results Act (GPRA) performance measures with the HHS strategic goals, and extrapolating from the cost of activities associated with those measures in order to prorate the entire NIH budget. The measures themselves are listed in the CJ, starting on page OA-40. NIH implemented a representative approach to GPRA reporting in response to OMB's mandate that the agency revise its comprehensive goals to bring them to a lower level of aggregation and to add greater specificity of desired outcomes. This representative approach was implemented in FY 2003. Since the GPRA performance measures represent a very small portion of the NIH budget (see table below), minor changes in the number, cost or budget mechanism of measures can cause major shifts in the distribution of the overall NIH budget by strategic goal. The FY 2015

budget was a transition year in terms of the number of GPRA measures and cost (both of which decreased); this shift amplified the impact of changes to the measures on the budget distribution.

For example, a single GPRA measure has been associated with Goal 1 (Strengthen Health Care) for the past three years, and the cost of this measure declined from \$49.5 million in FY 2014 to \$46.3 million in the FY 2015 President's Budget -- a decrease of nearly 6.5 percent. However, since the total extramural dollars for GPRA measures affiliated with other goals decreased by 41.5 percent in FY 2015, the dollars assigned to Goal 1 became a larger portion of the sample. When extrapolated to the entire NIH budget, this caused the estimate for Goal 1 to increase from \$1.003 billion to \$1.612 billion. As a result, the increase did not reflect any change in resource allocation, just a long-standing methodology that has become unreliable due to changing circumstances.

There was also an increase in the estimate for Goal 4 (Increase Efficiency, Transparency, and Accountability of HHS Programs) from \$1.011 billion to \$1.581 billion. Most of the change resulted from the completion of one Goal 2 (Advance Scientific Knowledge and Innovation) measure that had \$14.9 million attributed to the Research Management & Support (RMS) mechanism. Removing that measure from the sample increased the percentage of GPRA measures associated with Goal 4 among the measures with RMS funds, thus causing a shift from Goal 2 to Goal 4 of \$418 million in extrapolated RMS funds at the NIH level. There was no intent to estimate a reduction of NIH's emphasis on Goal 2 in FY 2015.

For FY 2016, NIH intends to revisit the methodology for this budget exhibit in order to reflect the distribution of budget dollars by HHS Strategic Goal as accurately as possible.

| Fiscal Year | FY 2013 | FY 2014 | FY 2015 |
|---------------------------------|--------------------------------------|--------------------------------------|--------------------------------------|
| Number of Measures with funding | 30 (25 in Goal 2) | 25 (20 in Goal 2) | 24 (17 in Goal 2) |
| Cost of those Measures | \$1.65 billion | \$1.31 billion | \$0.89 billion |
| Total Budget | \$29.15 billion (17.7 multiplier) | \$30.15 billion (23.0 multiplier) | \$30.36 billion (34.1 multiplier) |

25. Secretary CDC Funding

The 2015 CDC request overview identifies "Increased Program Investments" that represent strategic choices. Please provide the detailed criteria and resource allocation method used by CDC and the HHS Secretary to select these investment suggestions for Congress to consider. For each initiative, please provide the specific strategic linkage and the specific short and long-term performance measure for each for FY 2014 through FY 2019. Plus identify the specific

justification and expected change in performance measures for all the activities listed in the section “Program Decreases and Eliminations.”

As the Nation’s public health experts, CDC leadership works with the Secretary and others in the Administration to select investments that will positively impact public health throughout the Nation and the world. According to OMB Circular A-11, the Executive Branch communications that led to the President’s budgetary decisions will not be disclosed either by the agencies or by those who have prepared the Budget. The nature and amounts of the President’s decisions and the underlying materials are confidential.

CDC’s Congressional Justification provides detailed resource requests and justifications for all funding levels, including increases, decreases, and level funding. Information about the proposed allocations’ effect on performance is incorporated into targets found in the Performance section of CDC’s CJ, found on pp. 336-416.

For several increases proposed, CDC has provided targets beyond FY 2015, should requested resource levels be appropriated and maintained:

- The Antibiotic Resistance initiative, at a funding level of \$30 million for each of the next five years would:
 - Reduce *Clostridium difficile* (deadly diarrheal illness that kills up to 14,000 Americans per year) by 15%, preventing at least 1,600 deaths, over 13,000 hospitalizations, and over \$170 million in healthcare costs;
 - Reduce *Carbapenem-resistant Enterobacteriaceae* (CRE) infections (the nightmare bacteria with up to a 50% mortality rate) by 10%, drug-resistant *Pseudomonas* by 6% (some strains resistant to nearly all antibiotic therapies), invasive MRSA by 6% (80,000 infections and 11,000 deaths per year), and drug-resistant *Salmonella* infections by 5% (100,000 infections per year);
 - Expand drug susceptibility testing for high-priority pathogens by at least five-fold, including a ten-fold increase in the number of resistant *Salmonella* isolates from humans available for comparison with *Salmonella* isolates from animals and foods;
 - Begin work on an antibiotic resistant isolate library that will be accessible to pharmaceutical companies testing new antibiotic agents, biotech and diagnostic companies designing the next generation of clinical tests, and researchers evaluating the effectiveness of new interventions;
 - Begin implementation of CDC antibiotic protection tools nationwide to improve prescribing in U.S. acute-care hospitals and outpatient settings.
- The Global Health Security program, at a funding level of \$45 million for each of the next five years, would contribute to the US Government goal of working with at least 30 countries to effectively protect at least 4 billion people against the threat of infectious disease epidemics, and to engage with other countries around the world with the goal of protecting all 7 billion people.

26. Mine Safety

Please explain how CDC expects to protect its work in underground mine safety as it has closed its underground testing facility and does not have any specific plans in the FY 2015 request to re-establish this activity. Plus, provide a revised timeline on when the capability will be re-established through a new operational mine.

With the closure of the Lake Lynn Experimental Mine, NIOSH continues to carry out research projects in the areas of mine explosion, dust exposure and fire suppression as far as they can be taken in a laboratory setting, consistent with work since 2008. Virtual reality technologies at the Pittsburgh Research Laboratory are being used for training of mine rescue teams and for development of mine rescue operation procedures.

27. CDC Global Polio Eradication

Is the goal of global polio eradication and certification by 2018 achievable? Please provide an update on the performance measures related to the achievement of that goal with the most current status of the indicators. Identify what resources – by the international community, private sector donors, and the U.S. Government – are being used and required to meet this goal?

Yes, the goal of global polio eradication and certification in 2018 is achievable if external events do not derail progress. Forty-nine of the 62 cases of polio reported worldwide as of April 23, 2014 are from Pakistan. Nigeria has seen a marked decrease in the number of wild poliovirus type 1 (WPV1) cases, from 122 in 2012 to 53 in 2013, with only three cases in the past six months. Nearly eighteen months have passed without detection of wild poliovirus type 3 (WPV3) anywhere in the world. The WPV1 outbreak in the Horn of Africa appears on the verge of cessation, with the latest reported case occurring in January. Despite these successes, an outbreak in Syria has worsened the country's humanitarian crisis, has resulted in a case in Iraq, and is threatening the polio-free status of the other surrounding countries; the WPV1 outbreak in Cameroon has spread to Equatorial Guinea and risks further spread; and Southern Afghanistan, where the length of time between cases appeared to have suggested interruption of endemic transmission, reported its first indigenous WPV1 case since November 2012.

Afghanistan: Afghanistan continues to make steady improvement to its program. For the past year, polio cases in Afghanistan occurred almost exclusively in the Eastern Region and were linked to importation from Pakistan. These importations will continue as long as there is polio in Pakistan. However, they have not resulted in re-establishing persistent transmission in Afghanistan. The occurrence in December of an endemic WPV1 case in the Southern Region after 13 months of absence reveals continued surveillance gaps. Environmental surveillance recently began at selected sites in Kandahar city and will soon be expanded to other Regions.

Pakistan: For the past year, a large, uncontrolled outbreak of WPV1 continued in the Federally Administered Tribal Areas (FATA), with virus spread throughout areas of Pakistan and eastern Afghanistan. Poliovirus transmission will continue in both countries until vaccination in North Waziristan can be resumed and the outbreak stopped. Violence against health workers has continued, resulting in the deaths of dozens of polio workers and security officers assigned to protect them. Since early this year, with the strong political, security and logistical backing of the government of Khyber Pakhtunkhwa (KP) province, the program was able to deliver seven million doses of vaccine over twelve weeks in Peshawar without any attacks on health workers. Lessons learned from this experience are being applied elsewhere in KP and in Karachi. Refusals in Pakistan have been reduced by 80% in 2013; Pakistan now has the lowest rate of refusals in the world. New polling data shows significant social trends in FATA, however, that point to elevated distrust in Oral Polio Vaccine (OPV), frontline workers and the program more than any other high-risk area.

Nigeria: The program in Nigeria is more coherent and effective than ever before. The program is well coordinated, innovative and adaptive. Improvements in Kano state since late 2013 have been particularly striking. For the past six months, wild poliovirus appears to have been isolated to Kano and Borno States. Assuming the program can maintain improvements in Kano, the major endemic polio reservoir for Nigeria, the state will no longer be the source of wild poliovirus importations into West and Central Africa. Borno state continues to face substantial security challenges and has continued gaps in surveillance that it is attempting to address. While access to children has improved substantially during the past year, access continues to be limited in many areas and supplementary immunization activity (SIA) quality remains inadequate in areas that are accessible. Overall commitment to OPV (the proportion of parents stating that OPV is a “good” or “very good” idea for their children) is over 95% overall in the Northern states but is substantially lower in Borno. A major risk on the horizon is waning political commitment during the upcoming election season.

Syria and Iraq: Since polio cases were first discovered in Syria in mid-October, the program has mounted a regional response, coordinating efforts to boost immunity in all surrounding countries as well as Egypt, the West Bank and Gaza, while responding to the outbreak in Syria itself. Within Syria, vaccination has occurred throughout much of the country, including in contested and most opposition-controlled areas. Case counts have been low, but surveillance is clearly inadequate, although improving. The only known exportation since the discovery of the outbreak was to Iraq, where a series of SIAs was already in progress.

Central Africa: Since mid-November 2013, Cameroon has been responding to an outbreak caused by a WPV1 that had been circulating undetected somewhere in the region, probably for more than two years. The occurrence of cases two months later and the geographic spread within Cameroon and to Equatorial Guinea show that surveillance and SIA quality improvements are needed. The program is now (in early May) accelerating efforts to address this issue. In the meantime, Equatorial Guinea, with a

poor immunization system and little experience with polio SIAs, has been slow to respond. However, as of mid-April, the government has been very intensively engaged and at a very high level. Surveillance there is clearly inadequate, which suggests the outbreak is already widespread within the country. CDC has worked with the State Department to raise the profile of outbreak response with the governments of Chad, Central Africa Republic, Cameroon, Equatorial Guinea, Congo, and Democratic Republic of Congo, promoting a coordinated regional response.

Horn of Africa: There have been no cases in this outbreak since January 5, suggesting that virus transmission may have been interrupted. If so, the outbreak, affecting Somalia, Kenya and Ethiopia, will have been contained within 8 months of its discovery despite severe operational constraints, particularly in the South-Central Zone of Somalia. The most recent case was reported from Ethiopia, which had been slow to address program deficiencies in its Somali Region.

Israel, the West Bank and Gaza: From August through October last year, Israel mounted a national bivalent OPV (bOPV) campaign and followed with a 2nd-dose campaign limited to the south of the country. From shortly after the campaign began, environmental surveillance showed the virus becoming progressively more localized to a few communities around Bersheva, in southern Israel. In early March 2014, environmental sampling turned negative for a single week for the first time in over a year, although some subsequent specimens have been positive. In Israel, bOPV has been incorporated into the routine immunization schedule, which had previously exclusively relied on IPV. The West Bank and Gaza already include OPV in a sequential IPV-OPV immunization schedule.

Vaccine supply: Global supplies of both OPV remain constrained through the middle of 2014, after which projections show that supply will exceed demand.

Financing: Against the US \$5.5 billion budget for 2013–2018, the best-case funding gap for the entire period is US \$563 million. Many of the pledges made by donors at the 2013 Abu Dhabi polio summit meeting remain to be fulfilled. As of April 2014, the Global Polio Eradication Initiative (GPEI) had cash-on-hand of US \$721 million against a total 2014 budget of US \$1.033 billion.

The spearheading partners of the GPEI are the World Health Organization (WHO), Rotary International, CDC, and UNICEF. CDC deploys a wide range of public health assistance in the form of staff and consultants, provides specialized laboratory and diagnostic expertise and contributes direct funding. Below is a table which summarizes the funding for GPEI's Financial Resource Requirements (FRR) 2013-2018. In addition to the contributions toward the FRR displayed below, CDC also directly supports research and the global polio laboratory network. CDC's scientists conduct polio research around the world, collaborating with a wide range of partners. The results from this research helps guide policy and strategies and identify strategies best practices towards the GPEI endgame.

**Summary of Confirmed Funding Against the Global Vaccine Summit Commitments
(US dollars in millions)**

| | FUNDS COMMITTED BY APRIL 2013 VACCINE SUMMIT | CONFIRMED FUNDING AGAINST THE GPEI FRRS, AS OF FEBRUARY 2014 |
|---|---|---|
| United States ¹ | 90.60 | 129.85 |
| G8 & European Community Countries | 868.43 | 435.95 |
| Non-G8 Organisation for Economic Co- operation and Development Countries | 294.73 | 54.86 |
| Other Donor Countries | 15.56 | 15.47 |
| Private Sector/ Non-Governmental Donors | 2334.56 | 586.49 |
| Multilateral | 379.77 | 254.43 |
| Domestic Resources | 58.20 | 40.01 |
| TOTAL | \$4,041.85 | \$1,517.06 |

¹ The FY 2014 appropriation for CDC polio activities was \$151 million and \$38 million for USAID polio activities. The FY 2015 President's Budget requests \$161 million for CDC and \$38 million for USAID polio activities.

28. Sign up vs. Enrollment

It is my understanding that the enrollment numbers the White House is using, 4.2 million is the total number of people who have selected an insurance policy – not the number of people fully enrolled or who have paid for it.

The actual number of those who pay for insurance is forecasted to be much lower – reported to be around 20-30% of people who selected a plan did not make their first payment.

At current levels, it takes enrollment from 4.3 million down to about 2.9 million. Even if the White House meets its new goal of 6 million, actual enrollment would be closer to 4.2 million.

How will this impact the financial footing of Obamacare?

We are pleased with the number of Americans who have already enrolled in coverage through the Health Insurance Marketplaces, and are expecting to see a spike in enrollment as we move into the final two weeks of open enrollment. We are confident that we will receive sufficient enrollment through the Federally-facilitated Marketplaces.

29. Outreach budget request

The FY15 POTUS budget request plans to filter \$1.829 billion more into the federal exchanges (add new figures) and \$1.9 billion more into the state exchanges.

The federal portion includes \$774 million for “consumer information and outreach.” As the deadline for enrollment is March 31, 2014, why do you need \$774 million for consumer information and outreach next year? How much of this is going to be used for commercials like Broinsurance? What are the cost of these commercials?

The FY 2015 President’s Budget requests \$774 million for consumer information and outreach activities, of which \$703 million will be funded through user fees. This funding will primarily go toward operating the Marketplace call center and in-person assistance, with smaller amounts for enrollee notices and an outreach media campaign. Ongoing outreach and assistance is necessary since we expect significant new enrollment in the Marketplaces in the coming years. In addition, existing enrollees may experience changes in circumstance that change their eligibility for programs and subsidies, and they will need access to resources at the call center and Navigators to best assess their coverage options. In FY 2013, CMS obligated \$77 million for the consumer outreach campaign for the initial open enrollment period.

30. Relationship between HHS and IRS on ObamaCare

Between 2010 and 2012, the Department of Health and Human Services (HHS) transferred \$488 million from the Health Insurance Reform Implementation Fund to the Internal Revenue Service (IRS) for the IRS’s administrative expenses. The transfer was accomplished using an allocation account, allowing IRS to obligate up the amount in the allocation account.

The Centers for Medicare & Medicaid Services (CMS) is now paying the advance payment of the premium tax credit (APTC) from an IRS allocation account.

Why is CMS paying the APTCs? Why isn’t the IRS paying the APTCs? What other Federal agencies outside of the IRS are paying tax credits? How much and how frequently is the allocation account funded? Does CMS request amount for the allocation account? What is the review process for making this request?

How long does it take IRS to respond?

How often and at what level of detail does the CMS report to the IRS about the obligations made against the allocation account?

How much does it cost the CMS to administer the payments? Does the allocation account cover these costs as well?

What happens when CMS pays an APTC for a person that an insurer does not recognize as a policy holder? Can the insurer refuse those funds? How the CMS collect those funds? Are they deposited back into the allocation account? What is the process for resolving the overpayment?

What happens when an insurer does not receive an APTC for a policy holder that is eligible for an APTC? Does the insurer report the missing APTC to the policy holder or to the CMA? What is the process for resolving the missing payment?

Determinations of advance payments of the premium tax credit (APTC) are the responsibility of the Marketplaces, following HHS' rules and guidance. The source of funding for the premium tax credit, including the advance payment of the premium tax credit (APTC), is a permanent, indefinite appropriation to the Secretary of the Treasury for the payment of refunds and refundable tax credits. IRS manages and administers this appropriation on behalf of the Secretary of the Treasury. As the "parent" of the Treasury account, the IRS ensures sufficient budget authority is available at the beginning of the fiscal year and that sufficient funding has been transferred into the "child" allocation account for CMS use in obligating and disbursing funds for timely APTC payments. CMS uses its own administrative resources for its costs to make APTC payments.

The payment process occurs on a monthly basis for the APTC payments. A payment file is created using payment data provided by insurers of the qualified health plans (QHPs) in the Marketplace and is uploaded into HIGLAS, which is CMS' accounting system of record. Upon funds certification by a CMS certifying officer, CMS will submit information, with respect to the APTC payments to be made to the issuers of the QHPs, to the Secretary of the Treasury for payment. The Bureau of Fiscal Service, on behalf of the Secretary of the Treasury, makes the APTC payments to the issuers of the QHPs. Each month, financial accounting data is automatically sent to the IRS from HIGLAS. This data extract contains the accounting information/account balances (e.g., funds available, obligations, and disbursements) that the IRS is required to capture in their accounting system and report in their financial statements since they are the "parent" of the Treasury account used to make the APTC payments.

31. Premium Tax Credits

- **When did the IRS begin paying premium tax credits?**
- **How much has been paid to date?**
- **How much will be paid by the end of fiscal year 2014?**

- **How many households have the Federal and State healthcare exchanges reported to IRS as qualified for a premium tax credit to date?**
- **How many of those households elected to receive the advance premium tax credit?**
- **How many income and family size changes have been reported to the IRS to date?**
- **What happens when an insurer receives an advance premium tax credit for someone who, according to the insurer's records, is not a customer of theirs? How does the IRS collect the funds from the insurer?**
- **What happens when an insurer does not receive an advance premium tax credit for someone who is eligible for the credit and enrolled in an exchange plan? How does the IRS assist that person?**

Premium tax credits will be claimed and advance payments of the premium tax credit will be reconciled for the first time on taxpayers' 2014 federal income tax returns filed with the IRS in 2015. Accordingly, premium tax credits have not yet been processed by the IRS.

Based on data for the first five months of the initial open enrollment period, more than eight out of ten (83 percent) of the people who have selected a Marketplace plan through the SBMs and FFM are eligible to receive Federal financial assistance in paying their premiums. For the SBMs, 81 percent of enrollees selected Marketplace plans with financial assistance (10-1-13 to 3-1-14). For the FFM, 85 percent of enrollees selected Marketplace plans with financial assistance (10-1-13 to 3-1-14).

CMS is using an interim process to make payments of advance premium tax credits to insurers for eligible customers. This process is based on reports of confirmed enrollments from issuers and, therefore, issuers will not receive APTC payments for individuals who are not their customers.

32. Uninsured Americans:

How many Americans did the Administration assume were uninsured prior to the implementation of ObamaCare? Further, how many Americans does HHS assume will be uninsured as of the March 31, 2014 enrollment date?

The Administration remains focused on increasing access to health coverage to millions of Americans through the new avenues of coverage available through the Marketplace, expanded Medicaid, and by ensuring that individuals would not be discriminated against in the private insurance market because they had a pre-existing condition or be charged more for health coverage in non-grandfathered plans based on their health status or

gender. Each year, the United States Census Bureau releases estimates on the number of insured Americans as a part of the American Community Survey.

33. Title 42

Please update all the tables and dates provide in the fiscal year 2013 HHS Secretary hearing questions for the record for the questions under the title “Continued Excessive Use of Special Title 42 Pay Authority.” The update should add a column for fiscal year 2012, FY 2013 actuals and projected for fiscal year 2014 and FY 2015.

See attached.

34. Improve Recovery Audit Contractor (RAC) Program

Please provide a timeline and the specific steps that CMS and OMHA are jointly taking to implement a process across all operations to increase its focus on preventing improper payments and paying claims right the first time. Plus, the specific steps to reduce the CMS RAC program incentives to take overly aggressive actions by the RAC. Specifically, identify how CMS is working the stakeholders to evaluate the program’s data to identify challenges and make reforms. Finally, provide an update on the steps HHS is taking to establish a systematic feedback process with the OMHA, CMS programs, and the RACs to prevent the appearance that RACs are selecting determinations to increase their fees.

HHS continues to make improvements to the Recovery Audit Program to ensure the accuracy of Recovery Auditor determinations and to promote transparency within the program, and has moved beyond initial planning to working with agency staff to further develop various options. The development of options is still pre-decisional, but we have identified a wide array of potential actions. HHS is working through the details before making any public announcements as to courses of action in order to minimize confusion. HHS will keep the Committee updated on the status once we arrive at a plan and move forward.

35. Reduce Duplication Across HHS

Please provide a copy of the performance measures and process the HHS Secretary reviews to monitor and reduce duplication across HHS programs and OpDivs. Please provide the specific date of the Secretary’s last review and the schedule for the FY 2014 reviews.

The annual budget process is the Department’s primary method to identify and eliminate redundancy and duplication across programs. The process begins in the spring of each year, when HHS operating divisions are required to submit budget justifications, which include detailed budget and performance measures information, to the Assistant Secretary

for Financial Resources. Those justifications undergo rigorous examination, which includes review by the Secretary's Budget Council. Once Departmental decisions are finalized, revised justifications are submitted to the Office of Management and Budget. The result is a streamlined budget request to Congress, which provides critical investments in health care, disease prevention, social services, and scientific research in order to create healthier and safer families, stronger communities, and a thriving America.

In addition, as part of the Administration's Executive Order on efficient spending, HHS reviewed categories of administrative spending to find ways to improve efficiency and lower cost. For example, HHS was able to achieve savings in printing and reproduction by shifting printed material to digital and online access, and reducing hard copy printing.

36. HHS Communication Activities

Please provide a table that displays how much each HHS Office of the Secretary Office and OpDiv spent on communication and public relations related activities for each year from FY 2012 through FY 2015 projected (by OpDiv and year). Further, please explain the last time HHS conducted an internal review that included an aspect of how to best reduce duplication and ensure all activities specific related to core mission requirements. If such a review was conducted in the past 3 years, please provide a copy of such report.

The HHS Office of the Secretary and HHS operating divisions are responsible for promoting transparency, accountability and access to critical public health and human services information to the public, media, and constituency groups. Many of the Department's communications efforts are embedded in agency operating budgets and program operations, so a breakout of HHS-wide communications activity is not available in the format requested.

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lower cost. For example, HHS was able to achieve savings in printing and reproduction by shifting printed material to digital and online access, and reducing hard copy printing.

37. HHS Administrative Costs

In the March 15, 2013 Questions for the Record response, HHS identified that “as part of the Administration’s Executive Order on efficient spending, HHS reviewed categories of administrative spending to find ways to improve efficiency and lower cost”. Please provide a copy of each category reviewed, the potential savings available, and the amount that was included within each OpDivs or HHS Office of the Secretary’s office account for FY 2013, 2014, and 2015 budget development processes.

Data for the Executive Order on Efficient Spending is not available at the office account level. The Executive Order instructed agencies across government to reduce spending in FY 2013 for certain administrative categories by 20 percent. As part of this exercise, the Department reviewed administrative spending associated with travel, printing, employee IT devices, executive motor fleet, conferences, supplies, and management support services. Examples across the Department include developing and monitoring conference plans, consolidating mobile device accounts, and employing webinars and teleconferencing, and reductions in procurements. As a result, the Department estimates reduced administrative spending in these categories of approximately \$864 million in FY 2013 compared to a FY 2010 baseline. The Department will continue to focus on reducing administrative spending in the current year, as well as FY 2015.

38. CDC Winnable Battles

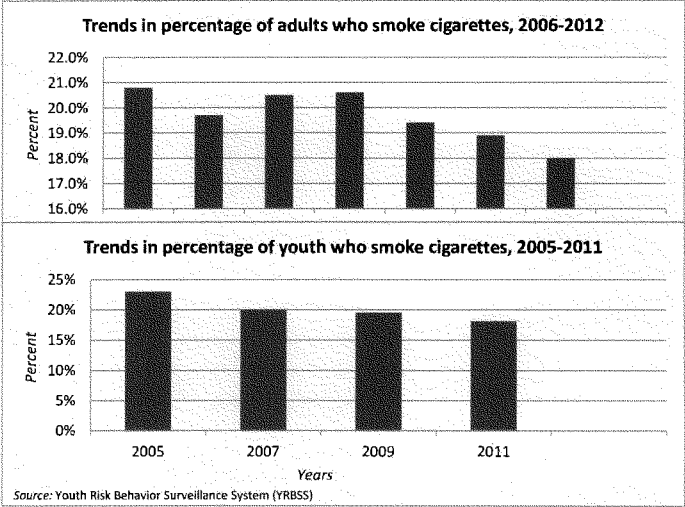
Please provide the base line measures for each CDC Winnable Battle and the annual performance measures reviewed by the CDC Director and his Center Directors to monitor activity in these areas.

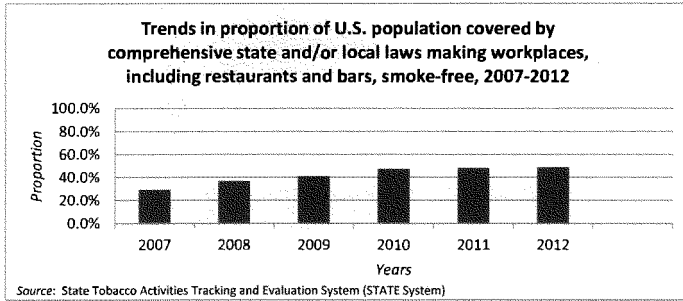
The baseline and annual performance data for the measures associated with CDC’s seven Winnable Battles are listed below. These performance measures assist the CDC Director and his Center Directors in monitoring progress in these areas.

Tobacco

Prevent the initiation of tobacco use, promote quitting, and ensure smoke-free environments.

| INDICATOR | BASELINE | MOST RECENT RESULT |
|---|--------------|--------------------|
| Decrease the percent of adults who smoke cigarettes | 20.6% (2008) | 18.0% (2012) |
| Decrease the percent of youth who smoke cigarettes | 20.0% (2007) | 18.0% (2011) |
| Increase the proportion of the U.S. population covered by smoke-free laws | 36.7% (2008) | 48.9% (2012) |

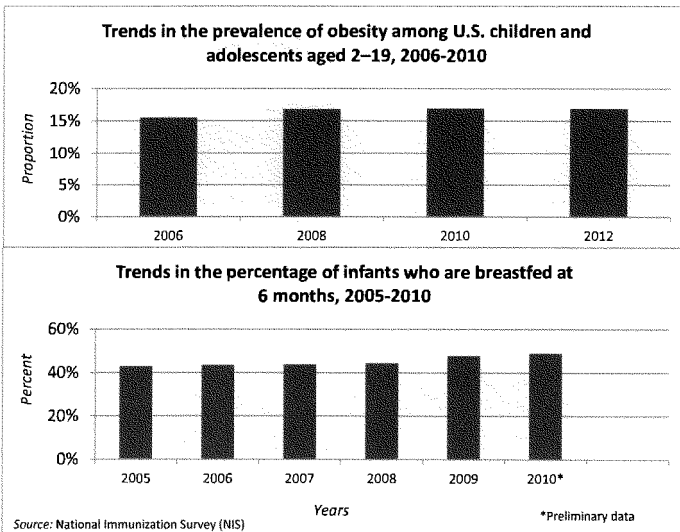




Nutrition, Physical Activity, and Obesity

Support all Americans in achieving optimal health by making nutritious foods and physical activity easy, attractive, and affordable.

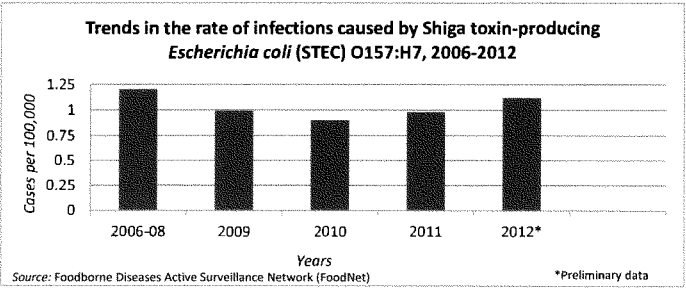
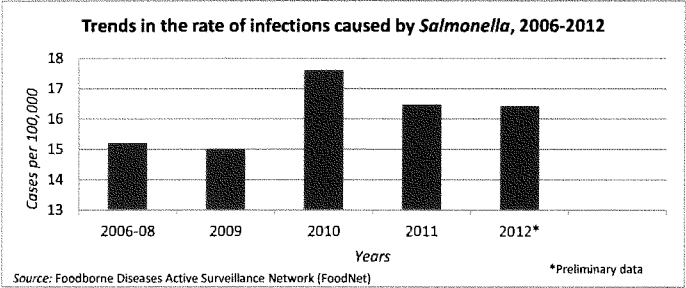
| INDICATOR | BASELINE | MOST RECENT RESULT |
|--|-------------------|--------------------|
| Reduce the proportion of children and adolescents age 2-19 who are obese | 16.8% (2007-2008) | 16.9% (2010) |
| Increase the proportion of infants who are breastfed at 6 months | 43.5% (2006) | 49.0% (2010) |



Food Safety

Keep America’s food supply safe by preventing and responding to foodborne illness.

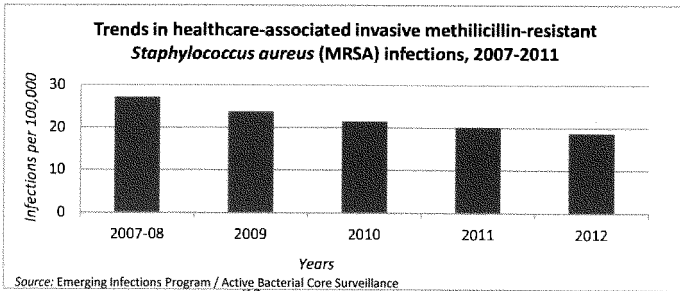
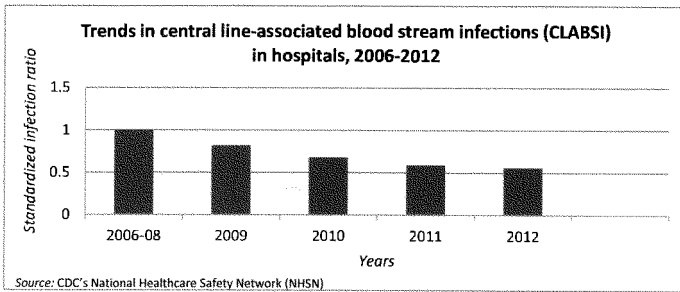
| INDICATOR | BASELINE | MOST RECENT RESULT |
|---|---|---|
| Reduce the rate of laboratory-confirmed infections caused by <i>Salmonella</i> | 15.2 cases per 100,000 population (2006-2008) | 16.42 cases per 100,000 population (2012) |
| Reduce the rate of laboratory-confirmed infections caused by Shiga toxin-producing <i>Escherichia coli</i> (STEC) O157:H7 | 1.2 cases per 100,000 population (2006-2008) | 1.12 cases per 100,000 population (2012) |

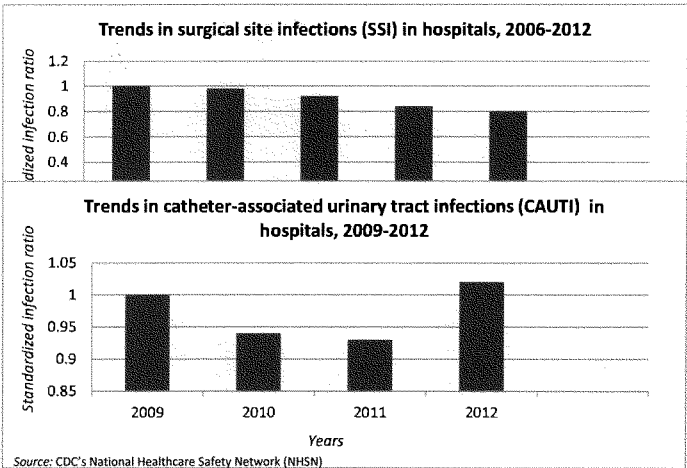


Healthcare-Associated Infections (HAI)

Ensure healthcare for all Americans by eliminating healthcare-associated infections.

| INDICATOR | BASELINE | MOST RECENT RESULT |
|---|--|--|
| Reduce central line-associated blood stream infections (CLABSI) in hospitals | 1.0 (2006-2008) | 0.56 (2012) |
| Reduce healthcare-associated invasive methicillin-resistant <i>Staphylococcus aureus</i> (MRSA) | 27.08 infections per 100,000 persons (2007-2008) | 20.06 infections per 100,000 person (2011) |
| Reduce surgical site infections (SSI) in hospitals | 1.0 (2006-2008) | 0.80 (2012) |
| Reduce catheter-associated urinary tract infections (CAUTI) in hospitals | 1.0 (2009) | 1.02 (2012) |

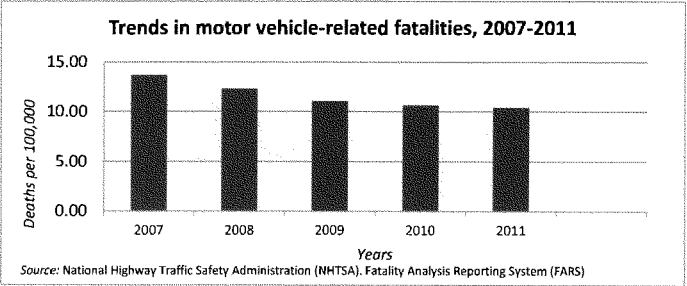




Motor Vehicle Safety

Keep people safe on the road – everyday.

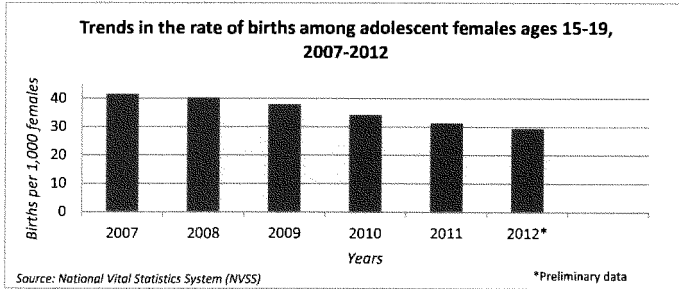
| INDICATOR | BASELINE | MOST RECENT RESULT |
|--|---|--|
| Reduce fatalities due to motor vehicle crashes | 13.8 deaths per 100,000 population (2007) | 10.39 deaths per 100,000 population (2011) |



Teen Pregnancy

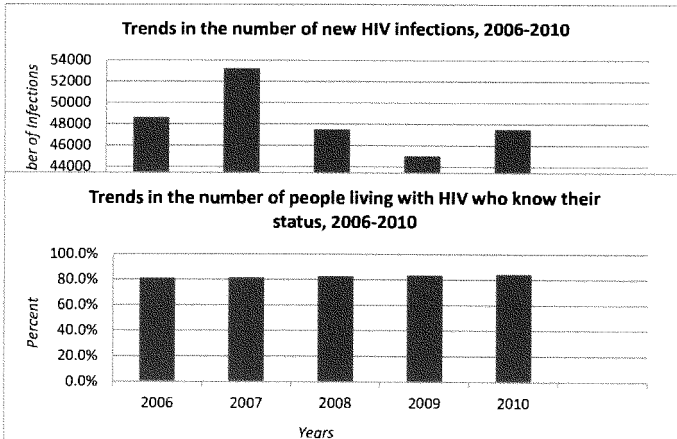
Reduce teen pregnancy and its contribution to the cycle of poverty for teens and their families.

| INDICATOR | BASELINE | MOST RECENT RESULT |
|---|--|--|
| Decrease teen birth rate among adolescent females ages 15 to 19 | 37.9 births per 1,000 females ages 15 to 19 (2009) | 29.4 births per 1,000 females ages 15 to 19 (2012) |

**HIV Infection**

Prevent new HIV infections and ensure quality health care for persons living with HIV.

| INDICATOR | BASELINE | MOST RECENT RESULT |
|--|--|--|
| Reduce the number of new HIV infections | 48,600 new HIV infections (2006) | 47,500 new HIV infections (2010) |
| Increase the percent of people living with HIV who know their status | 80.9% of people living with HIV know their status (2006) | 84.2% of people living with HIV know their status (2010) |



39. CDC Community Guide

The FY 2014 CDC request included a specific request for funding of this program that was not funded in FY 2014. We believe that no funds are being used for this program in FY 2014, is that correct?

The Community Preventive Services Task Force (Task Force) is an independent, nonfederal Task Force whose work is outlined in Section 399U of the Public Health Service Act, and compiled in the Guide to Community Preventive Services (Community Guide). CDC is required to provide "...ongoing administrative, research, and technical support for the operations of the Task Force..."

In FY 2013, the Community Guide was supported by \$6.99 million from the Prevention and Public Health Fund and \$1.465 million from budget authority for Public Health Scientific Services. In FY 2014, CDC plans to support the Community Guide with \$4.8 million from budget authority for Public Health Scientific Services.

40. Medicare Hearing and Appeals Rate

Please provide a table that breaks out for each of the past five year the number of improver claims identified through any program that that were appealed at any level in the process, the overturn rate at each step, and the value of the overturned rate. Please provide a separate breakout for all improper payments identified through the RAC process with the appeal rate for each step, dollar value, and percent overturned at each step in the process for each of the past five years.

The tables below show overpayment determination and appeals data for the first two levels of appeal for Medicare Fee-for-service claims reviewed by both Recovery Audit Contractors (RACs) and other review contractors. RAC data for 2013 will be made available in the 2013 RAC Report to Congress.

| LEVEL ONE REVIEW - MAC Claims Decisions | | | | | | |
|---|---------------------|----------------------------|-----------------------------------|-----------------|---------------------|-------------------------------------|
| Type | Fiscal Year Decided | Claims Denied ² | # of Claims Appealed ⁴ | Fully Favorable | Partially Favorable | Adjudicated Reversal % ¹ |
| Non-RAC | 2009 | 134,777,215 | 2,704,890 | 1,241,817 | 81,919 | 69.12% |
| | 2010 | 131,419,681 | 2,592,512 | 1,101,541 | 80,158 | 53.03% |
| | 2011 | 128,540,179 | 2,759,502 | 1,196,737 | 98,824 | 53.05% |
| | 2012 | 149,472,137 | 3,231,950 | 1,248,362 | 87,054 | 45.96% |
| | 2013 | 141,867,231 | 3,498,155 | 1,144,660 | 119,335 | 39.35% |
| | | | | | | |

| Type | Fiscal Year Decided | Adjustments Received ⁴ | # of Claims Appealed ³ | Fully Favorable | Partially Favorable | Adjudicated Reversal % ² |
|--|---------------------|-----------------------------------|-----------------------------------|-----------------|---------------------|-------------------------------------|
| RAC | 2009 | \$2,910 | 1,612 | 1,057 | 2 | 64.30% |
| | 2010 | \$323,305 | 20,847 | 12,725 | 452 | 68.18% |
| | 2011 | \$918,080 | 106,291 | 42,924 | 4,175 | 47.00% |
| | 2012 | \$1,473,413 | 279,455 | 76,843 | 5,994 | 30.54% |
| | | | | | | |
| ¹ Does Not Include Dismissals | | | | | | |
| ² DATA SOURCE: 2009-2011: Contractor Information Management System, CMIS; Bills Not Approved (Part A) and Claims denied including non-CWF claims/2012-2013: CROWD | | | | | | |
| ³ CROWD; RAC Data not reported prior to April 2009 | | | | | | |
| ⁴ RAC Data Warehouse - Overpayments Only | | | | | | |

| LEVEL TWO REVIEW - QIC Decisions ¹ | | | | | |
|--|---------------------|----------------------|-----------------|---------------------|-------------------------------------|
| Type | Fiscal Year Decided | # of Claims Appealed | Fully Favorable | Partially Favorable | Adjudicated Reversal % ² |
| Non-RAC | 2009 | 36,662 | 5,116 | 1,653 | 18.9% |
| | 2010 | 63,089 | 3,195 | 3,838 | 11.4% |
| | 2011 | 67,379 | 5,931 | 2,202 | 12.4% |
| | 2012 | 122,779 | 6,080 | 3,400 | 8.0% |
| | 2013 | 124,389 | 11,828 | 3,206 | 13.8% |
| RAC | 2009 | 14,463 | 7,905 | 248 | 60.0% |
| | 2010 | 341 | 119 | 3 | 38.7% |
| | 2011 | 8,017 | 1,301 | 185 | 18.9% |
| | 2012 | 80,448 | 13,188 | 972 | 17.9% |
| | | | | | |
| ¹ Represents claims received on overpayment decisions only | | | | | |
| ² Does Not Include Dismissals | | | | | |
| DATA SOURCE: AdQIC, received 6/2/14; QIC Decisions between 10/1/08 and 9/30/13 | | | | | |

41. CDC Prevention Research Centers

In the FY 2014 budget CDC noted that if funded at the FY 2014 request level it would fund 32 Prevention Research Centers, down from 37. In FY 2014, the enacted level was equal to the FY 2014 request level. Please provide a list of the 32 centers funded with FY 2014 funds.

If CDC did not fund 32 centers in FY 2014 as was proposed and approved in the FY 2014 enacted appropriations act, please provide the specific justification and explanation why this is contra to the requested policy.

Further, if this program was not executed within the scope of the budget policy, please provide a detailed report for each CDC program that will not be implemented in accordance with the FY 2014 proposed budget policy scope and detailed justification as to why it is inconsistent with the budget justification.

The Budget contains CDC's best estimates and projections at the point in time that it is published. After the projections for the Prevention Research Centers (PRCs) program were published in the FY 2014 President's Budget, CDC gathered stakeholder input on the new five-year funding opportunity announcement with schools of public health, state health departments, and local health departments. Two main recommendations came out of this process: (1) make larger awards to enhance the effectiveness of PRCs (meaning fewer awards would be made) and (2) ensure a strong link between PRCs and state, local and/or tribal health departments.

The funding opportunity announcement released on July 9, 2013 incorporated both of these recommendations, identifying that awards would be made to 25-30 grantees, and requiring evidence of strong linkages (e.g., letters of support, memoranda of understanding) to state and local health departments. Twenty-six awards will be made, at the end of May, in line with both the funding opportunity announcement and the objective review panels' recommendations. Once awarded, the list of awardees will be posted at http://www.cdc.gov/prc/center-descriptions/index.htm?s_cid=cs_732.

42. NIH Proposed Expanded Undiagnosed Diseases Network (UDN)

The NIH proposed expanded Undiagnosed Diseases Network (UDN) is proposed to handle an increase in the value of the significant public investment in the creation and operation of the Network. We understand the National Center for Biotechnology Information (NCBI) would maintain the NIH databases to optimize data analysis and public access to the data.

Please provide the underlying assumptions for the current program and FY 2015 proposed expanded UDN pilot program:

- **Specific purpose of each program**
- **Funding Source for each program**
- **Funding Levels (FY 2013, 2014, 2015) for each program**
- **Anticipated maximum annual cost of the proposed expanded UDN program**
- **Anticipated date and source of funding for the UDN program**

- Business case and funding mechanism of the proposed UDN expanded pilot as it relates to the NCBI cost and operations
- Scope of each program
- Capacity of each program
- Measures of success for each program
- Liability of current program on federal government and liability related to use by providers, patients, and federal government for the proposed UDN expanded pilot
- Length of the proposed expanded UDN pilot
- Criteria to evaluate the success of the proposed expanded UDN pilot
- Cost to establish the proposed expanded UDN pilot
- How will the data for the proposed expanded UDN pilot be captured, used, and provided in a usable format to private providers to research and diagnosis patients
- Explain how the proposed UDN expanded pilot anticipates ensuring a secure open access database and how it will link to other federal resources
- How will this method of data deposition amongst numerous outlets help physicians who are caring for undiagnosed cases when they don't know what it is they are looking for or where/how to look for it
- What evidence supports that this is the most efficient and effective mechanism to assist physicians in a single resource to identify and match other undiagnosed cases with the one that they are caring for
- Once a private sector provider identifies a match, what additional assistance will NIH provide and how will this be funded by NIH
- What is the limit on NIH's assistance to provide providers who access the database with clinical support, expertise, and other resources to help them diagnose and improve the care and outcome of their patient? Further, how will NIH fund these activities?
- How much does NIH expect to collect through the use of its 3rd party collections pilot for this program?
- Please provide the percentage assessment for each ICs contribution for the current program?

Answer: The UDN seeks to improve the level of diagnosis and care for patients with undiagnosed diseases, facilitate research into the causes of undiagnosed disease, and create an integrated and collaborative research community to identify improved options for patient care and treatments. The Undiagnosed Diseases Program (UDP), which has been seeing patients since 2008 at the NIH Clinical Center, is a part of the UDN as one of the clinical sites where undiagnosed patients are seen, and, therefore, has the same objectives.

- Funding Source for each program

Answer: The UDN is funded through the NIH Common Fund. As part of the UDN, the UDP receives UDN funds through the NIH Common Fund

■ Funding Levels (FY 2013, 2014, 2015) for each program

Answer: The UDN received \$10.6 million for FY2013. Funding for FY 2014 is \$19.6 and \$29.7 million is anticipated for FY2015.

■ Anticipated maximum annual cost of the proposed expanded UND program

Answer: The maximum anticipated budget in current out-year plans is \$30.8 million for FY 2016, subject to the availability of sufficient appropriations.

■ Anticipated date and source of funding for the UDN program

Answer: The UDN is funded through the Common Fund and began receiving support in FY 2013

■ Business case and funding mechanism of the proposed UDN expanded pilot as it relates

Answer: See below.

■ to the NCBI cost and operations

Answer: Existing NCBI databases and infrastructure are used for making UDN data accessible to researchers and clinicians. These databases are built and maintained for the specific purpose of storing data from many sources. Thus, there is no additional cost for NCBI hosting data generated by the UDN. Some UDN funding may be used to create additional databases if determined to be necessary, through the UDN Coordinating Center at Harvard Medical School.

■ Scope of each program

Answer: The UDP is one clinical site of the UDN. In addition to the UDP site, 5-7 other clinical sites will be announced this summer. Collectively, the clinical sites will work together to see patients with undiagnosed diseases and conduct research toward finding a diagnosis. The UDN will provide improved patient access to state-of-the-art diagnostic methods, by expanding the available expertise and facilities serving patients with these unusual disorders, and it will accelerate discovery and innovation in diagnosing and treating these patients. In addition to the immediate needs of the patients seen through the UDN, the research conducted at the clinical sites to understand these devastating conditions will provide insights into the underlying causes of both rare and common diseases.

■ Capacity of each program

Answer: The UDN will ramp up gradually. The first UDN clinical site, the UDP at the NIH Clinical Center, currently sees approximately 130 patients each year. The remaining 5-7 clinical sites will ramp up gradually to see at least 50 patients per year per site by FY 2016.

■ Measures of success for each program

Answer: Success is measured in terms of advancements made in the scientific understanding of disease and health and in searching for answers for patients with mysterious undiagnosed diseases for whom finally having a diagnosis can be powerful. In quantifiable terms, the UDP has identified over 60 candidate genes and posted them on a website for other investigators to pursue in collaborations. The Program has made diagnoses of more than 70 extremely rare diseases, and has published more than 30 scientific articles.

■ Liability of current program on federal government and liability related to use by providers, patients, and federal government for the proposed UDN expanded pilot

Answer:

The informed consent at the existing UDN clinical site (i.e. the UDP at the NIH Clinical Center) acknowledges the Clinical Center will provide short-term medical care for any injury resulting from participation in the research project, but that no long-term medical care or financial compensation for research-related injuries will be provided by the National Institutes of Health, the Clinical Center, or the Federal Government. Similarly, once the awards are made to fund the other UDN sites, those sites will be expected to establish policies on the limits of the grantee institutions' liability.

■ Length of the proposed expanded UDN pilot

Answer: The UDN began in 2013 and is currently funded for seven years.

■ Criteria to evaluate the success of the proposed expanded UDN pilot

Answer: Please refer to the answer provided to question j.

■ Cost to establish the proposed expanded UDN pilot

Answer: Please refer to the answer provided to question c for funding levels in the first three years of the program.

■ How will the data for the proposed expanded UDN pilot be captured, used, and provided in a usable format to private providers to research and diagnosis patients

Answer: Important clinical insights learned in UDN cases, such as links between specific genomic variants and diseases, will be available on the appropriate NIH public databases. For example, links discovered between specific genomic variants and diseases will be made available through the ClinGen database (a database currently under development that will catalogue which variants in the human genome play a role in disease: <http://www.clinicalgenome.org/>) to provide guidance to

healthcare professionals regarding which variants have clinical relevance for patient care.

Additionally, part of the UDN budget will fund researchers conducting follow-up studies to investigate the variants found in UDN patients. The findings of these researchers will also be made available in the appropriate federal databases in accordance with NIH genomic data sharing policies.

- Explain how the proposed UDN expanded pilot anticipates ensuring a secure open access database and how it will link to other federal resources

Answer: All UDN research data captured in databases will be managed through rigorous data security practices in order to protect the privacy of the research participants. Important research findings will be provided to the community in a de-identified format through federal resources such as ClinVar (a database that aggregates information about sequence variation and its relationship to human health: <https://www.ncbi.nlm.nih.gov/clinvar/>) and ClinGen. In addition, the UDN may establish other databases and tools for data analysis that will comply with Health Insurance Portability and Accountability Act and Federal Information Security Management Act regulations regarding access and minimization of identifiable data. Patients will be informed during the research consent process that steps are being taken to minimize the risk to their privacy being violated, but that this risk cannot be completely eliminated.

- How will this method of data deposition amongst numerous outlets help physicians who are caring for undiagnosed cases when they don't know what it is they are looking for or where/how to look for it

Answer: The UDN specifically aims to develop tools and outlets that engage as many researchers and clinicians as possible in order to build an integrated and collaborative research community. The Network will perform outreach to identify cases of undiagnosed patients, and to provide a portal for physicians to submit applications to the UDN on behalf of their patients. Furthermore, the UDN will develop methods and collate research discoveries to guide the diagnosis of patients presenting with mysterious diseases. These will then be shared and used by clinicians and researchers outside the program to help even more patients.

- What evidence supports that this is the most efficient and effective mechanism to assist physicians in a single resource to identify and match other undiagnosed cases with the one that they are caring for

Answer: The original UDP pilot has demonstrated that it is possible to establish a research study recruiting patients with mysterious diseases through physician submitted enrollment applications to advance methods of diagnosis and our knowledge of rare diseases. A limitation of the UDP has been the capacity of the program to see patients in the NIH Clinical Center; hence, the UDN was established.

The UDN will enable the collective capacity of these institutions to be pooled for the pursuit of diagnoses of more patients, as well as the development of resources that can be shared with the larger medical and scientific communities.

- Once a private sector provider identifies a match, what additional assistance will NIH provide and how will this be funded by NIH

Answer: The UDN will share the expertise and resources for researching undiagnosed diseases across the country by creating geographically diverse clinical and research centers that can engage with more patients. Therefore, private physicians who have patients they believe to have undiagnosed diseases may be able to collaborate with the researchers and physicians at the UDN clinical sites directly. These activities are encompassed within the UDN mission and will be supported through the UDN grants and NIH intramural program as funds permit.

- What is the limit on NIH's assistance to provide providers who access the database with clinical support, expertise, and other resources to help them diagnose and improve the care and outcome of their patient? Further, how will NIH fund these activities?

Answer: UDN seeks to gain knowledge of how to seek a diagnosis and determine the causes of disease. Knowledge may then be shared with providers through databases and academic articles for distribution to professional societies and other continuing education groups. Specific knowledge gained about individual patients in the UDN will be communicated to their referring physicians. This sharing of knowledge gained through research is fundamental to NIH's mission. Therefore, although the NIH does not provide clinical support or care, it can empower other providers to give better care based on improved knowledge. These activities are encompassed within the UDN mission and will be supported through the UDN grants and NIH intramural program as funds permit.

- How much does NIH expect to collect through the use of its 3rd party collections pilot for this program?

Answer: For the extramural sites, the program awards grants to these institutions and does not collect any funds. Individual clinical sites may, however, choose to collect 3rd party reimbursement for medically indicated testing. For the intramural UDP site, funds are not collected from 3rd party payers at this time.

- Please provide the percentage assessment for each ICs contribution for the current program?

Answer: The project is funded directly by the Common Fund.

43. National Cancer Institute's Frederick

The February 28, 2014 Cancer Letter that the National Cancer Institute's Frederick National Laboratory for Cancer Research is slated for an increase in Fiscal 2014 over its nearly \$300 million Fiscal 2013 budget. Please provide the FY 2011, 2012, 2013, 2014, and 2015 budget for NCI Frederick National Laboratory?

If there are any proposed increases for the Frederick National Laboratory, what are the research initiatives and projects planned for that facility?

How do those research initiatives and projects coordinate with NCI's extramural research projects and centers that will be awarded in Fiscal 2014 and beyond?

Does NIH anticipate any overlap with NCI funded research conducted at our nation's research institutions and that conducted by researchers operating at NCI's Frederick National Laboratory?

Q1: The February 28, 2014 Cancer Letter that the National Cancer Institute's Frederick National Laboratory for Cancer Research is slated for an increase in Fiscal 2014 over its nearly \$300 million Fiscal 2013 budget. Please provide the FY 2011, 2012, 2013, 2014, and 2015 budget for NCI Frederick National Laboratory?

A1: At the outset, it is important to understand the relationship between the Frederick National Lab for Cancer Research (the Frederick National Lab, or FNLCR) and the Federally Funded Research and Development Center (FFRDC). As authorized by the Federal Acquisition Regulations, NCI's FFRDC operates under a broad charter to enhance the ability of NCI to achieve its mission and to rapidly, flexibly, and efficiently conduct and support cancer research. The Frederick National Lab includes the scientific research performed at a dedicated site in Frederick Maryland with contract employees. In addition to conducting scientific research for the Frederick National Lab, the FFRDC contractor (Leidos Biomedical Research Inc., or LBR) also provides essential support for the Frederick National Lab and provides other support for NCI programs, such as executing subcontracts and supplying technical and infrastructural assistance for programs run by the various NCI divisions.

The table below displays NCI's total FFRDC obligations for FY 2011, FY 2012, and FY 2013 and obligations to date for FY 2014. For FY 2014 and 2015, NCI expects that the FFRDC obligations will be similar to funding levels for previous fiscal years. In addition to the total FFRDC obligations, we are also displaying the subset of obligations that represents the funding for laboratory research performed in Frederick that is conducted by LBR employees.

The funding displayed for FFRDC obligations for FY 2012 show an increase of \$61.3M compared to FY 2011. In large part, this increase was driven by funding from the Division of Cancer Treatment and Diagnosis to launch the Clinical Assay Development

Center (CADC) and the NCI Experimental Therapeutics (NeXT) program. Completing the fit-out and relocation to the new Frederick Advanced Technology Research Facility (ATRF) also contributed to the FY 2012 increase in FFRDC obligations. The funding displayed for FY 2012 within the subtotal line of the table show a \$31.1M increase compared to FY 2011, and this increase also relates to the launch of the CADC and NeXT programs, as well as obligations related to the ATRF.

NCI FFRDC Obligations
Dollars in Millions

| | FY 2011 | FY 2012 | FY 2013 | FY 2014* |
|---|-----------------------|-----------------------|-----------------------|----------------------|
| FFRDC Obligations | \$297.9 | \$359.2 | \$368.8 | \$150.0 |
| <i>Subtotal, Frederick LBR Lab Component</i> | <i>\$136.8</i> | <i>\$167.9</i> | <i>\$159.1</i> | <i>\$64.7</i> |

* Obligations through March 2014

Q2: If there are any proposed increases for the Frederick National Laboratory, what are the research initiatives and projects planned for that facility?

A2: NCI does not have plans for FY 2014 budget increases. We expect that the FY 2014 funding will be similar to the funding level for FY 2013.

Q3: How do those research initiatives and projects coordinate with NCI's extramural research projects and centers that will be awarded in Fiscal 2014 and beyond?

A3: The broad scope of the FFRDC and the scientific programs of the Frederick National Lab are designed to complement the NCI science programs conducted through extramural grants and contracts developed by several of the NCI divisions, and through NCI's intramural programs.

The Federal Acquisition Regulations (FAR) offer important program flexibility for NCI's FFRDC. However, the FAR expressly prohibits FFRDC contractors from performing work that could be done as effectively through other government mechanisms, such as grants, other contracts, and cooperative agreements. This standard also applies to work of the Frederick National Lab conducted through the FFRDC. The FAR limitation helps assure coordination so that FFRDC activities do not duplicate or compete with work conducted by NCI grantees and contractors. NCI also has review processes to support this limitation.

NCI directs and oversees the work of the FFRDC and the Frederick National Lab in consultation with the NCI-Frederick Advisory Committee (NFAC), a committee chartered under the Federal Advisory Committee Act, and in consultation with other

advisory boards. This helps ensure the optimal use of the Frederick National Laboratory in meeting the needs of the NCI and the cancer research community. NCI is currently leading a process in close collaboration with its advisors to leverage the Frederick National Lab and the unique qualities of the FFRDC to accelerate discovery and translation of scientific findings.

Examples of specific, coordinated initiatives and projects that the Frederick National Lab will support during FY 2014 and beyond include the AIDS and Cancer Virus Program, the Antibody Characterization Program, the Biopharmaceutical Development Program, the Basic Sciences Program, the Core Genotyping Resource, and the Nanotechnology Characterization Laboratory. These laboratory programs are conducted in response to requirements from – or in collaboration with – NCI intramural or extramural investigators.

Q4: Does NIH anticipate any overlap with NCI funded research conducted at our nation's research institutions and that conducted by researchers operating at NCI's Frederick National Laboratory?

No, we do not foresee any substantial overlap. Of course, as is true for all funding mechanisms, there are commonalities of purpose (e.g., to understand the generic basis of cancer or develop new cancer therapeutics), but important efficiencies are achieved based on the approach used in a given funding mechanism. As the response to question 3 indicates, the FFRDC offers a unique and flexible capability for NCI to achieve its mission. At the same time, sections of the FAR prohibit FFRDC contractors from performing work that could be done as effectively through other government mechanisms. This is designed to assure that the FNLRC complements, rather than competes with or overlaps, research that NCI funds through grants or other contracts.

Two specific examples of collaboration with universities are (i) the RAS Initiative and (ii) the Nanotechnology Characterization Laboratory.

(i) NCI recently launched a nationwide effort involving the Frederick National Lab to develop effective treatments for some of the most resistant cancers, including colon, lung, and pancreas cancers, driven by RAS oncogenes. NCI established the RAS Initiative without adding new funding to the Frederick National Laboratory during FY 2013 to harness recent advances to target tumors driven by mutant RAS. Much, although not all, of the work of the RAS Initiative occurs within the Frederick National Lab. The initiative uses a hub-and-spoke model to connect researchers at NCI's Frederick National Laboratory to a nationwide network of academic and industry collaborators, most of whom are already engaged with RAS-related projects. Leaders of the initiative, working at the Frederick National Lab, will coordinate the efforts of scientists from around the country, with support from NCI intramural and extramural funds, to apply new expertise and technologies to re-energize efforts to develop RAS therapeutics. To further the goals of the RAS Initiative, NCI convened an international workshop in Frederick to identify strategies for new drug targets in RAS-dependent cancers, which we expect will lead to

new NCI grant opportunities for academic scientists. NCI has scheduled two additional workshops on approaches to targeting RAS-driven cancers for the summer of 2014.

(ii) The Nanotechnology Characterization Laboratory (NCL) is a contractor-operated partnership between NCI, the U.S. Food and Drug Administration, and the National Institute of Standards and Technology. The partnership is designed to accelerate the safe and effective development of nanotechnology-based cancer treatments. NCL's services are available to researchers from any organization—academia, business, or government. Data generated from the characterization of nanomaterials can support investigational new drug or investigational device exemption applications.

NCL is located in the Frederick National Lab as part of the NCI's Alliance for Nanotechnology in Cancer. The Alliance began in 2004 as a comprehensive, systematized effort to engage the public and private sectors in multidisciplinary research designed to advance basic scientific discoveries and translate them into clinical applications. The Alliance provides grant support for team science across multiple laboratories, standardized formats, collaborations among academic, private, and public sector organizations, and high-throughput technology platforms.

Questions from Congressman Andy Harris

- 1. If the law requires that the insurance plans must list abortion services, why does the summary of benefits for plans offered in the state of Maryland remain completely silent on the question of abortion coverage, leaving the consumer completely in the dark about whether their plan covers abortion? During the hearing, you agreed that there should be transparency.**

CMS is committed to ensuring that HealthCare.gov provides the key information consumers need to make an informed selection from among the Qualified Health Plans (QHPs) available to them. The Affordable Care Act requires that each plan in the Marketplace include a Summary of Benefits and Coverage and a link to the plan brochure, where consumers can learn more about which services are covered.

- 2. What steps are you taking to ensure that both state exchanges and the federally facilitated marketplace are clearly identifying which plans include elective abortion?**

Consistent with section 1303(b)(2)(E) of the Affordable Care Act, state health insurance commissioners, or the appropriate state regulators, are responsible for collecting and reviewing issuers' plans related to segregation of funds. HHS does not plan to direct state insurance commissioners or other state officials in their efforts to enforce the requirements of section 1303.

- 3. What is the penalty for plans that fail to disclose this information?**

Consistent with section 1303(b)(2)(E) of the Affordable Care Act, state health insurance commissioners, or the appropriate state regulators, are responsible for collecting and reviewing issuers' plans related to segregation of funds. HHS does not plan to direct state insurance commissioners or other state officials in their efforts to enforce the requirements of section 1303.

- 4. Since this information is not being consistently disclosed to consumers, will you provide a list of subsidy-eligible plans on the state exchange and the federally facilitated exchange indicating whether the plan includes abortion (in cases other than the Hyde exceptions) as a covered benefit, and if so, please provide the amount of the separate abortion surcharge for that plan? Will you provide this information no later than April 30, 2014?**

CMS is committed to ensuring that HealthCare.gov provides the key information consumers need to make an informed selection from among the Qualified Health Plans (QHPs) available to them. The Affordable Care Act requires that each plan in the

Marketplace include a Summary of Benefits and Coverage and a link to the plan brochure, where consumers can learn more about which services are covered. The Affordable Care Act requires plans in the Marketplace to cover the ten essential health benefits. It is up to the issuer to determine which additional services they cover, and consumers may always contact issuers with any questions.

- 5. Statutory language in section 1303 of the Affordable Care Act requires that issuers of Exchange plans cover elective abortion must “collect from each enrollee in the plan” a “separate payment” for elective abortions (abortions in cases other than rape and incest) and a “separate payment” for all other services. The statute then stipulates that the insurer is to “deposit all such separate payments into separate allocation accounts.” In Maryland, the Insurance Commissioner issued a bulletin that specifically states “issuers are not required to provide enrollees with separate invoices for non-excepted abortion services [elective abortion] and all other services covered under a QHP [Exchange plan], nor to provide enrollees with itemization on a single invoice for non-excepted abortion services [elective abortion] and all other services covered under a QHP.” Has HHS directed plans to comply with the separate payment requirements? If so, please explain in what way? What actions do you plan to take to enforce that requirement and what actions can be taken to require states to comply with the law?**

CMS did not separately collect information about issuers' estimates of the actuarial value of coverage of abortion services for which public funding is prohibited. Rather, issuers were directed to include the costs attributable to abortion services for which public funding is prohibited with costs attributable to nonessential health benefits to facilitate the accurate display of premium information. Consistent with section 1303(b)(3)(B) of the Affordable Care Act, specified information including any advertising used by the issuer with respect to the plan and any information provided by the Marketplace must specify only the total amount of the combined charges for coverage of abortion services for which public funding is prohibited and for all other coverage provided by the plan.

Generally, HHS has sought to maximize the flexibility and discretion afforded to State-based Marketplaces within the parameters established by the Affordable Care Act. Consistent with this overall approach, HHS has not published specific guidance outlining how State-based Marketplaces should administer or oversee this specific statutory requirement. However, HHS has specified requirements in 45 CFR 156.280, which apply to issuers of all QHPs, including those in State-based Marketplaces. Additionally, 45 CFR 156.280(e)(2) specifically implements the “separate payments” requirement. State-based Marketplaces could provide additional direction to their QHP issuers if desired, provided that such direction is consistent with the statute and implementing regulations. Similarly, HHS recognizes that QHP issuers participating

in the Federally-facilitated Marketplaces may take any of several potential approaches to collecting these separate payments based on their administrative and business practices, also provided that such approaches are consistent with the statute and implementing regulations.

6. According to CRS report R41137, “In certain instances, the [premium tax credit] amount may cover the entire premium and the tax filer pays nothing toward the premium.” In such cases where the plan purchaser receives a 100% subsidy how does the insurance company collect the abortion surcharge described in 1303(b)(2)(i)(II) of the ACA?

The premium tax credit established under section 36B of the Internal Revenue Code may be used only to cover or reduce the costs of essential health benefits covered by a QHP. Further, the tax credit may not be used to cover the costs of abortion services for which Federal funding is prohibited, consistent with section 1303(b)(2)(A) of the Affordable Care Act. As described at 45 CFR 156.470, CMS implemented these requirements by collecting from issuers the portion of the total rate that is attributable to essential health benefits, excluding any costs attributable to coverage of services that are not essential health benefits, including abortion services for which Federal funding is prohibited. This amount is then used to calculate advance payments of the premium tax credit.

Pursuant to sections 1303(b)(2)(B)(i) and 1303(b)(2)(D)(ii)(III) of the Affordable Care Act, the issuer of a qualified health plan that provides coverage for abortion services for which Federal funding is prohibited must collect a separate, non-subsidized payment from each enrollee of an amount equal to the actuarial value of these services, which the issuer may not estimate to be less than one dollar per enrollee per month. Therefore, it is not possible for a consumer to have no out-of-pocket premium responsibility for a qualified health plan that covers abortion services for which Federal funding is prohibited.

Example: An issuer charges a rate of \$100 per month for a particular qualified health plan, of which one dollar is attributable to abortion services for which Federal funding is prohibited and four dollars are attributable to other non-essential health benefits. The premium tax credit eligible portion of the premium is \$95, meaning that the maximum amount of premium tax credit a consumer could apply to the plan, if eligible, is \$95 per month. Even if the consumer were eligible for a tax credit of \$97 per month, the consumer remains directly responsible for five dollars per month. On the Federally-facilitated Marketplace website, the consumer would be advised of his or her out-of-pocket responsibility (in this example, five dollars) during plan shopping.

7. For individuals who are eligible for cost-sharing credits, how will plans ensure compliance with section 1303(b) (2) (A) (ii)?

As discussed at 45 CFR 156.430 and finalized in the HHS Notice of Benefit and Payment Parameters for 2014, HHS will provide to QHP issuers advance payments and reconciliation payments based on cost-sharing reductions provided for essential health benefits, which do not include abortion services for which Federal funding is prohibited, see 45 CFR 156.280(d)(1). Further, in accordance with 45 CFR 156.280(e)(1)(ii), issuers must not use any cost-sharing reductions or advance payments thereof to pay for abortion services for which Federal funds are prohibited. Instead, claims for such abortion services must be paid out of the separate allocation account established for this purpose, see 45 CFR 156.280(e)(3).

8. Is abortion ever classified as a “preventive service” in plans sold on the federally-facilitated exchanges?

No.

9. According to a report entitled “Third Grade Follow-up to the Head Start Impact Study”, Head Start had little to no impact on cognitive, social-emotional, health, or parenting practices of participants. Specifically, the findings showed no statistically measurable effects on all measures of cognitive ability, including numerous measures of reading, language, and math ability. On a few measures, access to Head Start actually had harmful effects on children. Specifically, negative impacts included children in the third grade that had access to Head Start reported worse peer relations than their counterparts and their teacher-assessed math abilities were worse. The report was published by the Department in October of 2012 that you oversee. How can you continue to ask for additional resources given the findings of this report?

The Head Start Act, as amended in 1998, required the Secretary of Health and Human Services to study the program’s impact on children and families. In 2000, the Department commissioned the first large-scale randomized control trial of the national Head Start program from an independent contractor: the Head Start Impact Study. A report of interim findings was submitted to Congress in 2005 and a final report with findings through children’s first grade year was provided to Congress in January 2010. The third grade study was not required by Congress but was undertaken by ACF in order to understand longer-term impacts on children and families. This report, presenting findings through third grade, was completed in December 2012.

The Head Start Impact Study is nationally representative, including programs at all levels of quality; employs a randomized design; and examines all domains of children’s development and achievement as well as parenting through third grade. It examines the average impact of providing children access to one program year of Head Start at age

three or age four. It compares children randomly assigned to receive Head Start in 2002 to children who were denied Head Start but could – and often did – attend other programs. The study is unique from other studies of early care and education in that it includes a nationally representative sample, a randomized control design, and examines a comprehensive set of outcomes for children and families through 3rd grade.

As noted in the 2012 report and prior reports from this study, at the end of the initial year of study, Head Start had significant impacts in every domain examined. There were significant differences between the group given access to Head Start and the control group on every measure of children's preschool experiences measured in this study. These effects were found both for the 4-year-old cohort and for the 3-year-old cohort during the year in which they were admitted to Head Start.

The study also found significant favorable impacts of Head Start on child and family outcomes at the end of the first year. There were initial positive impacts of Head Start, for both age cohorts and across domains of development, including cognitive development, social emotional development, health, and parenting. However, by the end of 1st grade and again at 3rd grade there were very few impacts found for either cohort in any of the four outcome domains examined: cognitive, social-emotional, health and parenting practices. The few impacts that were found did not show a clear pattern of favorable or unfavorable impacts for children. However, the study included all children who were offered a Head Start slot, some of whom did not ultimately participate. As a result, the differences between the treatment and control groups are somewhat diminished.

While the Head Start Impact Study cannot speak to impacts beyond 3rd grade, it is still an important part of the larger body of evidence about Head Start's effect on longer-term outcomes. The Advisory Committee on Head Start Research and Evaluation concluded that both the Head Start and Early Head Start impact studies show immediate impacts on child and family well-being, and that while those immediate impacts do not persist into elementary school in the two impact studies conducted by HHS, other research suggests that longer-term impacts can still be found in adulthood. To support this conclusion, the Committee cited both evidence from non-experimental longitudinal studies of Head Start that have found beneficial effects into adulthood, as well as studies of other early childhood intervention programs that have found long-term impacts in adulthood despite diminished or no impacts during earlier follow-ups.

10. In FY 14, Head Start received a \$1.025B increase (FY13 \$7,573B and FY14 \$8,598B) and is requesting an additional \$270M in FY 2015. Why is the administration requesting an increase in funding when the agency's own scientifically rigorous evaluation found Head Start to be ineffective?

The evaluation did not find Head Start to be ineffective. As mentioned in the response to the previous question, the Head Start Impact Study found significant impacts in every domain studied at the end of the first year.

The FY 2014 increase supports the expansion of Early Head Start, including through Early Head Start–Child Care Partnerships, which will provide tens of thousands of our youngest and most vulnerable children with access to high quality comprehensive care. In addition, funding provided in FY 2014 funding will allow all Head Start and Early Head Start programs to keep pace with increasing costs without diminishing quality. The FY 2015 request maintains and builds on these important investments.

11. Last year the administration issued new regulations on Head Start, requiring underperforming grantees to apply through a competitive grant process for their Head Start funding. How is HHS assessing whether these grantees' performance has improved, and is HHS making that information about performance public?

ACF has launched an important research study titled Evaluation of the Head Start Designation Renewal System (DRS). This study will examine: 1) how effective the DRS is in identifying higher and lower performing Head Start programs, 2) how programs have understood and responded to the DRS in terms of improving program operations and quality, and 3) what competition looks like, and how programs are responding in communities where programs have been designated for competition. This evaluation of the DRS is being conducted independently from the Office of Head Start by the Urban Institute and the Frank Porter Graham Child Development Institute, University of North Carolina – Chapel Hill, under the sponsorship of the Office of Planning, Research and Evaluation in the US Department of Health and Human Services. Results from this study will be made widely available on ACF's website. As far as an individual grantee's performance is concerned, the new regulations allow us to use the competitive process to assess an applicant's performance through ongoing oversight by our Regional offices and periodic on-site reviews, which looks at all areas of performance including health and safety, fiscal and program management, and classroom quality through the Classroom Assessment Scoring System (CLASS). Beginning last year the Office of Head Start made all reports from monitoring reviews available to the public on our website.

12. In your budget, starting in 2018 you propose lowering the target rate for the Independent Payment Advisory Board (IPAB) from GDP per capita growth plus 1 percentage point to GDP plus 0.5 percent. This change cuts spending by 12.9B over 10 years. When will individuals be appointed to the IPAB? When do you believe IPAB will take effect?

The Independent Payment Advisory Board (IPAB) serves as a backstop to protect against excessive cost growth in the Medicare program. IPAB may not propose increases in cost-sharing or beneficiary premiums, restrictions on benefits, rationing of health care or changes in eligibility. Analysis conducted by the independent CMS Office of the Actuary

for the President's FY 2015 Budget projected that per capita Medicare spending growth will not exceed the statutory-based target specified for IPAB until 2019. The President's FY 2015 Budget, includes a package of legislative proposals that will save over \$400 billion over 10 years by more closely aligning payments with costs of care, strengthening provider payment incentives to promote high-quality efficient care and creating incentives for beneficiaries to seek high-value services. Enactment of these proposals would delay the date the target growth rate is exceeded.

13. Clinical data registries have met with the HHS Office of Human Research Protections and are waiting for that office to post guidance on applicability of the Common Rule to data collection by these registries. When can we expect to see this clarification?

Clarification of a series of issues related to the application of the HHS regulations for the protection of human research subjects, found at 45 CFR Part 46, to the development and operation of clinical data registries has been requested of the HHS Office for Human Research Protections (OHRP). For clarifications which do not require extensive review, OHRP plans to respond within the coming months. Additional clarification for issues which potentially involve regulation under the purview of the HHS Office for Civil Rights (OCR) and the Food and Drug Administration (FDA) have been submitted to the Secretary's Advisory Committee on Human Research Protections (SACHRP), an independent Federal Advisory Committee, for review. Specifically, the Committee has been tasked with offering its views on a series of issues regarding how certain regulatory requirements should apply to institutions collecting, sharing, combining, and analyzing large data sets for multiple purposes; and making recommendations to the Secretary. Those recommendations must be submitted to the Secretary; thereafter OHRP will work with OCR and FDA to provide guidance.

14. As I understand it, recipients of Primary Care Residency Expansion (PCRE) Program Grants will soon lose their funding for their fifth year of resident training. Secretary Sebelius, please describe the Department's plan to resolve this situation.

In FY 2010, the Health Resources and Services Administration awarded \$168 million in grants for the Primary Care Residency Expansion (PCRE) program using resources from the Prevention and Public Health Fund. Awards supported a fully funded project period of five years. While no funding is requested to continue this program beyond FY 2015, PCRE grantees may be eligible to compete for funding through the Targeted Support for Graduate Medical Education program, a new proposal in the FY 2015 President's Budget. The Targeted Support for Graduate Medical Education program would provide \$530 million in FY 2015 and \$5.2 billion over 10 years to support 13,000 medical residency positions across the country to train more physicians in primary care and high need specialties.

15. I am concerned with the National Cancer Institute's implementation of its plan to consolidate the clinical trial Cooperative Groups into the National Clinical Trials Network. The Cooperative Group Program represents a significant investment by American taxpayers over the years – an investment that has paid off in terms of improved outcomes for cancer patients and in lives saved. NCI acknowledged going into the reorganization that the NCTN would cost more money than the Cooperative Group system, at least in the short term. And yet, the \$25.6 million in additional funding that NCI had committed, and your Board of Scientific Advisors had approved for funding the groups is being spent elsewhere. In light of this fact, what will NCI do to ensure adequate funding and the continued robustness of this important clinical trials system that has provided the foundation for nearly every major advance in cancer patient care?

As you noted, NCI's major clinical trials enterprise, known as the Cooperative Group Program, has a solid record of accomplishment over many years. In 2010, the IOM, at NCI's request, examined the enterprise and issued a report that recommended systematic changes intended to help the NCI design, review, and conduct studies more efficiently. Acting on the IOM recommendations with extensive consultation with the Cooperative Group investigators and other components of our research and advocacy communities, NCI designed a new National Clinical Trials Network (NCTN) that builds on the success of the Cooperative Group Program and is designed to improve the speed and efficiency of cancer clinical trials.

In November 2011, the NCTN concept was presented to the NCI Board of Scientific Advisors (BSA), a committee that is chartered under the Federal Advisory Committee Act to provide scientific advice on a wide variety of matters, including evaluation of extramural program initiatives. The BSA approved the concept for the NCTN with a proposed budget of \$178 million for the first year, a \$25 million increase relative to the final year funding of the Cooperative Group Program. This increase was requested to provide consolidated services to the Network such as information technology (IT) resources, imaging and radiotherapy core services, and management resources for regulatory, administrative and tissue management. As in all such situations, actual spending was understood to depend on the NCI appropriations for FY 2014, the year in which NCI planned to launch NCTN. As you know, the FY 2014 appropriation for NCI was \$203 million less than the budget request, and this reduction significantly limited the ability of NCI to provide the funding increase recommended by the BSA.

NCI issued a series of Requests for Application (RFAs) for the components that make up the NCTN in July 2012; applications were received in January 2013; and the Network Group Operations Centers awards were made in April 2014. For the current fiscal year, FY 2014, the NCI appropriation is approximately 3 percent below the FY 2012 appropriation. NCI held the funding for clinical trials constant at the FY 2012 level of \$151 million for FY 2013 and FY 2014 to establish the NCTN. Based on these circumstances, NCI made the determination that a successful launch of the NCTN would

be best served by NCI investment in the new components of the network – consolidated infrastructure, imaging and radiotherapy core services, integrated translational science awards, and lead academic participating sites. These new components are essential to the improved approach to our nation's clinical trials network, with enhanced cooperation, consolidation, and flexibility as the NCTN's inherent attributes.

To enhance efficiency, the newly consolidated groups that comprise the NCTN are being asked to draw upon these core network resources rather than pay to establish their own services. To facilitate the transition to this new mode of operation and to explain the implications of these changes, senior NCI personnel are actively working with the Groups – speaking with the Group Chairs individually about their awards, and meeting with each of the Chairs and their key financial and statistical staff to discuss their specific concerns. NCI has also planned a meeting of all of the Group Chairs in the next few months to answer remaining questions.

16. Earlier this year, it was widely reported that you made fundraising calls for outside groups like Enroll America to encourage people to support groups who would be promoting the law. Was this action performed using any official resources?

Since the Affordable Care Act was signed into law on March 23, 2010, I have contacted two outside entities, neither of which is regulated by the Department of Health and Human Services (HHS), to ask that each of the two entities consider making financial donations to Enroll America. I made these calls in my capacity as Secretary of HHS. Implementing the Affordable Care Act is a very important part of the HHS Secretary's official responsibilities. Title XVII of the Public Health Service Act specifically gives the Secretary the authority to elicit this type of support.

17. MD's exchange is a disaster. The Inspector General has notified Chairman Kingston and me that it is going to be investigating it and as you know the GAO is looking into Oregon and some other states. MD has wasted over \$200M on their dysfunctional exchange. MD has a request in for an additional \$30M more for the exchange. Will HHS continue to send money to MD for it to waste on their failed exchange?

As Maryland works toward resolving technical problems with its Marketplace, the state and CMS will assess whether sufficient funding exists under its current award for these activities. If Maryland determines it is necessary to submit an application for additional Establishment Grant funding, CMS will review the request to ensure costs are allowable, allocable, and reasonable.

18. MD officials say they are not sure whether they will be able to keep their exchange after the March 31 deadline. Is your department ready to take over those responsibilities in MD and other states like Oregon and Hawaii where there have been major administrative or enrollment problems?

Consistent with prior years, the President's Budget projects long-term spending on an aggregate basis and does not project spending on a state-by-state basis. CMS is working with states on addressing the implementation challenges with their State-based Marketplace. States identified their functionality problems and implemented mitigation strategies under CMS guidance. CMS has put in place Corrective Action Plans for states that continue to have technical problems. Through these plans, states must demonstrate their ability to effectively oversee the implementation of a well-functioning Marketplace, including overseeing large contracts and holding vendors accountable.

19. When did you learn that the MD exchange was having problems?

I regularly receive updates on several aspects of implementation of the Affordable Care Act, including the status of the State-based Marketplaces.

20. Your budget proposes spending (mandatory) of \$1.899B in 2015 on exchange grants. How much of that money is going to go to MD? How much of it will go to other states that have dysfunctional exchanges?

Consistent with prior years, the President's Budget projects long-term spending on an aggregate basis and does not project spending on a state-by-state basis. As Maryland works toward resolving technical problems with its Marketplace, the state and CMS will assess whether sufficient funding exists under its current award for these activities. If Maryland determines it is necessary to submit an application for additional Establishment Grant funding, CMS will review the request to ensure costs are allowable, allocable, and reasonable.

21. Is the use of SIAs (Systems Improvement Agreements) accomplishing the goal to eliminate the Special Focus Facility (SFF) program?

The purpose of the Systems Improvement Agreements (SIAs) is not to eliminate the SFF program, but to encourage facilities to address root causes so that deficiencies are remedied and the remedies are sustained over time. The SIAs are designed to improve patient safety and health care outcomes by requiring systemic improvements on the part of the provider or supplier through an agreement between CMS and the provider/supplier.

The Systems Improvement Agreement is offered in very limited circumstances where CMS determines that:

- Termination of the provider or supplier would cause significant access to care issues or a significant disruption in care for Medicare or Medicaid beneficiaries (e.g., relocation of beneficiaries from their place of residence to locations away from nearby family).
- There are multiple, systems-level quality improvement efforts that would need to be undertaken not only to bring the provider back into compliance with Medicare requirements but also to ensure that such improvement are long lasting and sustainable.
- For nursing homes, it could include those facilities in the CMS Special Focus Facility program (SFF) that have track record of substandard quality care that have not addressed the underlying systemic problems that have given rise to repeated cycles of serious deficiencies (a “yo-yo” compliance history); and
- CMS’ offering of an SIA does not contravene any statutory or regulatory requirements.

The SFF program targets facilities that have had a serious history of persisting quality of care deficiencies. Many more facilities are in the SFF program than have SIAs. To the extent the SIAs can enable a facility to avoid a relapse back into subpar quality, the SIAs might reduce the extent of the need for the SFF program over time. However, CMS expects that there will be a continuing need for a robust SFF program given the large number of nursing homes that persist in offering poor quality of care. CMS believes this is one of the reasons that the Congress required the Secretary to maintain the SFF program and codified it in the statute in sections 1819(f)(8) and 1919(f)(10) of the Social Security Act, as amended by sections 6103(a)(3) and (b)(3) of the Affordable Care Act.

22. There are various indicators of the clinical performance of a skilled nursing facility, including CMS’s own Five Star System. Is that data considered when enforcement actions are taken due to a subpar survey?

Yes, in considering whether there are enforcement actions, CMS reviews the extent of non-compliance on a survey or series of surveys. The determinations of non-compliance may cover clinical quality of care issues as well as quality of life issues (resident dignity, personal funds, etc.). The CMS *Five Star Quality Rating System* incorporates the findings of the health surveys into the rating system.

23. Has the implementation of the SFF list increased quality?

An effectiveness evaluation of the SFF program conducted by Abt Associates for the years 2005-2011, shows an increase in quality for facilities in the SFF program. SFF facilities were found to come into compliance 50% more quickly than a comparison group of similar poorly- performing facilities. However, over time, SFF facilities also

tended to regress somewhat in their performance. Facilities in the comparison group of candidate facilities (who were notified of their risk of being selected as SFF facilities but were not selected) improved at a slower rate than the SFF facilities, but did not relapse as much as the SFF facilities once they were off the SFF candidate list. As a result of the evaluation, CMS is reviewing options for the SFF program intervention and will be pilot testing additional interventions in the coming year.

24. Are there standardized procedures for entry and exit from the SFF list applied uniformly in all states

Monthly, CMS provides each State with a list of candidates for the SFF program. This list ranks facilities, based on the results of their last three years of survey performance. The State may then choose any identified candidate on the list; or, with prior approval from CMS choose any nursing home within their state they determine to have a long history of poor performance. Additional procedures for the SFF program can be found at http://www.cms.gov/Medicare/Provider-Enrollment-and-Certification/SurveyCertificationGenInfo/downloads/SCLetter10_32.pdf.

25. Do you take into account changes in operators when you analyze removal from the list?

CMS does not take into account change of ownership or operators when creating the SFF candidate list. However a State may consider this information when selecting candidates from this SFF list. In addition, CMS and States will take change of ownership or change of operator into account when taking action relative to facilities that have not qualified to graduate within 18 months. Typically we expect facilities to improve sufficiently to graduate within 18 months. CMS may take change of owner or operator into consideration when determining the progressive enforcement actions that are applied for facilities that are failing to improve if there is evidence that the new owner or operator is making substantial efforts to transform facility performance.

Questions for the Record Ranking Member DeLauro

- 1. Please provide a more detailed explanation of the federal resources that are proposed in the FY 2015 budget request for the purpose of supporting federal and state health insurance exchanges in FY 2015. Earlier in the hearing, there was a suggestion that health insurance exchanges would cost more than \$23 billion next year, but I believe that figure includes a variety of costs and programs that are not, in fact, expenditures in support of health insurance exchanges. And I believe the vast majority of funds that were cited would be fully funded through offsetting revenues, for programs such as Transitional Reinsurance, Risk Adjustment, and Risk Corridors. Can you elaborate on the administration's request for funding to support health insurance exchanges? For instance, what is the total amount of taxpayer funding that would be expended to support these exchanges in FY 2015, and how does that compare to taxpayer funding that is projected to be expended in FY 2014?**

The only new cost for Marketplaces in FY 2015 that was not already paid for in the ACA, or financed by offsetting collections, is the \$629 million in discretionary Program Management funding, a decrease of nearly \$900 million below the FY 2014 President's Budget request.

Chairman Kingston cited a \$23 billion amount for Marketplaces in FY 2015, noting that this was an increase above the \$5 billion for FY 2014. The table above displays the programs included in the two totals. Characterizing the \$23 billion as additional Marketplace costs is inaccurate for several reasons.

1. Only the CMS Marketplace Funding and 1311 Marketplace Grants are directly related to Marketplace implementation. Total funding for these sources, including user fees, is projected to decline from FY 2014 to FY 2015.
2. Mandatory funding, such as the 1311 funding for state work to implement Marketplaces, was paid for in the Affordable Care Act through offsets to other federal spending, so this is not a cost.
3. The majority of spending increases in FY 2015 are related to programs that have offsetting collections. User fees, transitional reinsurance, risk corridors, and risk adjustment will be financed through offsetting collections.

| Activity | FY 2014 | FY 2015 | FY 2015 Collections | FY 2015 Net |
|-----------------------------------|--------------|---------------|------------------------|----------------|
| CMS Marketplace Funding | | | | |
| CMS Program Mgmt Appropriations | 611 | 629 | - | 629 |
| Other HHS funding/1 | 579 | - | - | - |
| User Fees | 200 | 1,200 | (1,200) | - |
| 1311 Marketplace Grants | 2,447 | 1,889 | - | 1,889 |
| <i>Marketplaces Subtotal</i> | <i>3,837</i> | <i>3,718</i> | <i>-1,200</i> | <i>2,518</i> |
| Mandatory Program Mgt Proposals/2 | - | 430 | - | 430 |
| PCIP | 952 | - | - | - |
| CO-OP | 296 | 157 | - | 157 |
| ERRP | 38 | 1 | - | 1 |
| Rate Review Grants | 80 | 50 | - | 50 |
| Transitional Reinsurance | - | 10,020 | (10,020) | - |
| Risk Adjustment | - | 3,378 | (3,378) | - |
| Risk Corridors | - | 5,450 | (5,450) | - |
| Total | 5,203 | 23,204 | -20,048 | 3,156 |

1/ Includes preliminary estimates for sources such as the Nonrecurring Expenses Fund and the Secretary's Transfer Authority

2/ The FY 2015 President's Budget included \$430 million for mandatory proposals unrelated to Federal Marketplace Funding. The largest of these proposals included \$400 million to implement the Medicare and Medicaid mandatory package.

2. I'm concerned about the administration's overreliance on competitive grants, which often result in cuts to important annual grants to state and local agencies. In its FY 2015 request, the administration proposes to cut \$1.4 billion in formula grants for programs such as the Low Income Home Energy Assistance Program, the Community Services Block Grant, the Preventive Health and Human Services Block Grant, and Public Health Emergency Preparedness Cooperative Agreements. Why does the administration continue to shift funds to competitive grant programs at the expense of base funding for state and local programs?

In developing the FY 2015 Budget, we made difficult decisions given the constrained budget environment to reduce discretionary funding in a number of areas while targeting investments in health care, disease prevention, social services, and scientific research, to support healthier families, stronger communities, and a thriving America. In some cases, the Administration has chosen to rely on competitive grants because they can help drive higher performance by states, localities, or other grantees and thereby improve outcomes for the populations they serve – including through the underlying formula funding.

The difficult decisions reference above included:

Low Income Home Energy Assistance: The Budget continues to request reduced funding, with decreases to the formula grant partially offset by the inclusion of a \$200 million contingency fund to respond to emergencies such as extreme weather and spikes in the price of fuels such as propane and a new \$50 million energy burden reduction grant to help cut heating costs for low-income households.

Community Services Block Grant: The Budget continues to request a reduced funding level and targets requested resources to maximize their impact in the communities it serves. Currently, this funding stream supports the important work of Community Action Agencies (CAAs), but does not hold these agencies accountable for outcomes, or target funding to the most effective agencies. The President's FY 2015 Budget targets resources to high-performing, innovative agencies and uses a three-pronged approach to increase accountability and local innovation in the program: 1) rewarding high performers; 2) using competition when programs fail to meet standards; and 3) authorizing the immediate suspension of funds in instances of fraud and criminal wrongdoing.

Preventive Health and Health Services Block Grant: The Budget continues to propose the elimination of this program because the activities it supports are better implemented through other mechanisms. These include the State Public Health Actions to Prevent and Control Diabetes, Heart Disease, Obesity and Associated Risk Factors and Promote School Health program, which provides resources to every state as part of the base formula to coordinate activities across categorical funding streams, as well as the Partnerships to Improve Community Health program and the Affordable Care Act Prevention and Public Health Fund investments.

Questions for the Record for HHS Secretary Sebelius **Submitted by Rep. Lucille Roybal-Allard**

Overmedication of children

In November 2011, Secretary Sebelius announced that ACF, CMS and SAMHSA would be taking steps to address the use of psychotropic drugs to treat children with behavioral problems. According to the GAO, these drugs represent the single largest expenditure in Medicaid—over \$2.8 billion in 2007.

Questions:

1. What concrete steps have been taken since that 2011 announcement?

HHS' Administration for Children and Families (ACF) monitors state compliance with federal child welfare requirements under titles IV-B and IV-E and the safety, permanency and well-being outcomes for children and families through the Child and Family Services Reviews (reviews). ACF has completed two reviews in every state since 2001 and is beginning a third round of reviews to occur during fiscal years (FYs) 2015 through 2018. As part of these reviews, ACF assesses the agency's efforts to support positive outcomes for children by using an onsite review instrument to review a sample of cases of children served by the state child welfare agency. ACF has always included an item in this instrument to consider a state's performance in assessing a child's mental and/or behavioral health needs and facilitating the receipt of appropriate services. ACF has revised the review instrument for the upcoming reviews to include a specific item on whether the agency provided appropriate oversight of prescription medicines for a child's mental health issues, including following the state's protocols for the appropriate use and monitoring of psychotropic medications. We believe this assessment will help provide information to states and help shape any further ACF guidance on addressing children in foster care's mental health needs appropriately.

Additionally, in FYs 2011, 2012, and 2013, ACF funded three successive cohorts of five-year discretionary grants focused on increasing children's psycho-social functioning, increasing permanency, improving adoption outcomes, and reducing unnecessary medication use by: 1) implementing universal mental health, behavioral health and trauma screening; 2) increasing access to trauma-informed and trauma-focused evidence-base interventions; and 3) introducing measurement-driven case planning, progress monitoring and service array reconfiguration.

ACF continues to actively partner with CMS and SAMHSA to reduce over-prescription of psychotropic medications for children in foster care by building State's capacity to prevent and mitigate the effects of trauma by supporting the development of: 1) service delivery capacity; 2) workforce and infrastructure; and 3) sustainable funding strategies.

Also, an ACF-led inter-agency work group is in the process of completing a guide for caseworkers, supervisors and caregivers on psychotropic medication (a companion to *“Making Healthy Choices: A Guide on Psychotropic Medications for Youth in Foster Care”* released in 2012 (available at <http://www.nrcyd.ou.edu/learning-center/med-guide>).

In July of 2013, ACF, SAMHSA and CMS issued a joint letter to state directors of child welfare, Medicaid, and mental health authorities encouraging them to strengthen their efforts to address complex trauma among children and youth known to child welfare. The letter provides useful and actionable information about federal authority and funding streams, strategies for coordinating cross-system efforts, and good practices for integrating evidence-based screening, assessment, and interventions related to complex trauma.

Finally, the FY 2015 President’s Budget includes a five-year CMS and ACF collaborative demonstration project to encourage states to implement evidence-based psychosocial interventions targeting children in the foster care system as an alternative to the current over-prescription of psychotropic medications in this population. This transformational approach would include the development and scaling up of screening, assessment, and evidence-based treatment of trauma and mental health disorders among children in foster care in order to reduce the inappropriate reliance on psychotropic medications. The agencies have been focusing on several evidence-based practices that are effective regardless of whether a child is living in a foster care family or in a therapeutic foster care family. We continue to examine models of therapeutic foster care as well, and are interested in the evidence base regarding the impact various models have on effective treatment for children.

2. Is it possible that Medicaid reimbursement policy encourages prescribing drugs rather than using alternative treatments that rely less on medicating the child?

Medical necessity in Medicaid is primarily a state determination, and certainly medications are a reimbursable service in every state. But particularly since the ACA, CMS has worked to support a wide variety of specific programs to support states in their efforts to best meet the needs of their populations using services beyond simply medications, including but not limited to:

- CMS in cooperation with SAMHSA and ACF have provided states with guidance and information regarding screening and interventions for children who experienced trauma, including children in foster care
- Health home authority in ACA has a strong focus on behavioral health, and states have chosen to focus on behavioral health of children
- Multiple pieces of guidance issued on integrated care models and shared savings, and institutions such as children’s hospitals have played a key role in some of these efforts

Recent rule (78 Fed Reg 42160 on July 15, 2013) allowing for broader use of the health workforce for purposes of prevention, including qualified professionals such as community health workers. No state has come to us with a proposal in this arena, but again, our work to issue the rule reinforces our commitment to providing states with the flexibility and tools necessary to tackle these kinds of programs.

CMS has also worked proactively with our partners to disseminate and provide technical assistance for these options. HHS partners have also been working in partnership with HHS to address the issue. For instance, the Medicaid Medical Director's Learning network partnered with AHRQ and CMS to examine and promote best practices to address the needs of children on antipsychotics.

3. Since the release of the GAO report, what new research, if any, has been undertaken to address this problem, and what percentage of the research is focused on looking for alternative treatments that would be used instead of, or in combination with, drugs?

SAMHSA is continuing efforts to address the use of psychotropic medications with children and youth who experience behavioral health problems. SAMHSA partnered with the Center for Health Care Strategies (CHCS) to provide educational webinars based on the Faces of Medicaid Data Analysis. This research was conducted to provide information about children's behavioral health service utilization on a sample of over 29,000,000 children (0-19 years of age) in the 2005 Medicaid data set. Results from this study revealed that 1.7 million children in Medicaid (6%) received psychotropic medications and that only 51% who were prescribed psychotropic medications received any behavioral health services.

SAMHSA is now extending educational opportunities by creating a Clinical Learning Community that will focus technical assistance efforts on developing alternatives to the use of psychotropic medications within grant communities and state children's behavioral health agencies.

Additionally, as part of the national evaluation of SAMHSA's Children's Mental Health Initiative (CMHI), SAMHSA is tracking the use of psychotropic medication and how services being delivered through a "system of care" framework are helping to reduce or avoid the use of psychotropic medications. Services delivered through a system of care approach can be used as an alternative to traditional services, including psychotropic medications, and this evaluation methodology will help SAMHSA develop and provide information about best practices in this area.

Mortality rates among mental health & addiction services patients

CDC and SAMHSA studies show that people with severe mental illnesses served by state mental health agencies die 25 years sooner than other Americans, mortality rates similar to those of people living in sub-Saharan Africa. Moreover, people receiving treatment through state alcohol and drug abuse programs also have very poor overall health status because of co-occurring chronic diseases, everything from HIV/AIDS and hepatitis C to cirrhosis and lung conditions.

Questions:

1. Are there any updates available about the implementation of the Primary Care Behavioral Health Integrations Grant program?

As you are aware, the Primary and Behavioral Health Care Integration (PBHCI) program supports community-based behavioral health agencies' efforts to build the partnerships and infrastructure needed to initiate or expand the provision of primary healthcare services for people in treatment for serious mental illnesses (SMI), co-occurring SMI, and substance use disorders. Since appropriations were first provided to SAMHSA in FY 2009, 100 grantees have received funding. In FY 2014, SAMHSA intends to award approximately 25 new PBHCI awards.

As of April 2014, nearly 50,000 clients have been enrolled in the PBHCI program. PBHCI grantees collect data on consumers at admission and in follow-up reassessments every six months, as well as at discharge when possible. Of those clients enrolled for a minimum of 6 (six) months, changes in health status were as follows:

| Measure¹ | Any Improvement² | No longer at Risk³ |
|----------------------------|------------------------------------|--------------------------------------|
| Blood Pressure | 18.5% | 17% |
| Body Mass Index | 43.4% | 4.6% |
| Waist circumference | 39.7% | 6.5% |
| Breath Carbon Monoxide | 32.7% | 6.9% |
| Plasma glucose, fasting | 35.7% | 10.6% |
| HgbA1c | 36.4% | 8.2% |
| HDL Cholesterol | 38.2% | 8.6% |
| LDL Cholesterol | 40.7% | 10.5% |
| Triglycerides | 39.8% | 11.4% |

With regards to the National Outcome Measures:

¹ This data is current as of April 2014, updating that which in the congressional budget justification.

² "Any improvement" includes even slight improvements in health status.

³ "No longer at risk" means the individual's health status fell below the designated risk threshold.

- **Health:** The percentage of consumers who rated their overall health as positive increased by 21.2% from baseline to most recent reassessment (N= 19,896).
- **Tobacco Use:** The percentage of consumers who reported they were not using tobacco during the past 30 days increased by 6.4% from baseline to most recent reassessment (N= 20,235).
- **Illegal Substance Use:** The percentage of consumers who reported that they were not using an illegal substance during the past 30 days increased by 10.5% from baseline to most recent reassessment (N= 18,208).

Social Connectedness: The percentage of consumers who reported positive social connectedness (having someone to talk to about problems, support from family or friends in a crisis) increased by 42.6% from baseline to most recent reassessment (N= 20,268).

2. What is the status of the Primary Care and Addiction Services Integration program?

The President's FY 2015 Budget proposes \$20 million for a new program, the Primary Care and Addiction Services Integration (PCASI) program. This program will enable substance abuse treatment providers to offer a full array of both physical health and substance abuse services to clients. The goals of this program will be analogous to the Primary Care Behavioral Health Integration (PBHCI) program discussed above.

The PCASI program would provide support integration and co-location of substance abuse treatment and primary care services at publicly funded centers and clinics. Development of PCASI is driven in part by the success of the PBHCI program and feedback from SAMSHA grantees and stakeholders that a similar effort was needed for patients with substance abuse disorders. Initially, 34 grants of \$500,000 will be provided to promote policy development, collaboration, staff training and organizational changes to improve overall patient health.

The PCASI and PBHCI grants will together help to promote a coordinated, integrated approach to behavioral health care.

3. Is the Department funding any other programs or research designed to address these mortality disparities in mental health and substance abuse patients?

HHS approaches mortality inequalities among patients with behavioral health conditions in several ways. SAMHSA collaborates, for example, with government agencies and other stakeholders extensively:

- In FY 2014, SAMHSA is implementing the Minority AIDS Initiative Continuum of Care Pilot. The goal of this effort is to encourage co-location of services and integration of care for patients with HIV/AIDS (<http://beta.samhsa.gov/grants/grant-announcements/ti-14-013>). This roughly \$17 million program is funded by SAMHSA's Centers for Mental Health Services, Substance Abuse Prevention and Substance Abuse Treatment.
- The Centers for Disease Control and Prevention's (CDC's) Million Hearts initiative focuses on decreasing heart disease morbidity and mortality, and SAMHSA contributes particularly for addressing patients with behavioral health conditions—a group that smokes tobacco at significantly higher rates than the population as a whole. (<http://www.integration.samhsa.gov/health-wellness>)
- SAMHSA in coordination with National Institute of Mental Health recently released guidance to states concerning a new set-aside required for the mental health block grant focusing on “evidence-based programs that address the needs of individuals with early serious mental illness, including psychotic disorders.” (See Guidance for Revision of the FY2014-2015 MHBG Behavioral Health Assessment and Plan, <http://beta.samhsa.gov/sites/default/files/mhbg-5-percent-set-aside-guidance.pdf>). Such early intervention efforts may help to improve long-term outcomes.

While these examples do not provide a comprehensive description, they point to the significant work that HHS does in the area of health disparities.

Congresswoman Barbara Lee – Questions for the Record

Diversity/Minority Health

The FY 2015 budget request:

- Proposes a 36 percent cut to the Office of Minority Health, from \$56.5 million enacted in FY 2014 to \$36 million in FY 2015;
- Eliminates funding for HCOP and AHEC;
- Eliminates funding for the Racial and Ethnic Approaches to Community Health program (REACH), once again; and
- While the NIH budget includes an increase of \$211 million over FY 2014 enacted levels (to \$30.4 billion), the budget proposes level funding for the NIMHD.

In response to inquiries regarding these cuts, the Secretary mentioned during the March 13, 2014 hearing that there was significant *new* spending on minority health initiatives. After further review of the budget, and upon confirmation from HHS staff, those new funds are in fact simply proposed increases to Health Center and Ryan White Part C-III funding.

While these programs are certainly an important element of the health care safety net and are of benefit to minority communities, they are not in any way a replacement or adequate alternative to the HCOP and AHEC programs, which are vital to building, recruiting, training and retaining a diverse health professions pipeline committed to the care of underserved populations; the REACH program, which is essential for the establishment of community-based programs and culturally-tailored interventions to eliminate health disparities; nor funding for the Office of Minority Health which is absolutely critical to the identification of racial and ethnic health disparities and the creation and implementation of programs to address those disparities.

Question 1: How does this Administration intend to fight health disparities, increase access, address health outcomes, and improve linguistically and culturally competent healthcare for minority communities while simultaneously de-prioritizing and eliminating the very programs designed to accomplish these goals in the budget, year after year?

HHS is dedicated to improving the health of all people and strives to emphasize diversity in all programs across the Department to focus on eliminating health disparities and achieving health equity. In FY 2015, HHS will make strategic investments in programs that will deliver the greatest impact on reducing health disparities in racial and ethnic minority populations across the country.

Within the Health Resources and Services Administration (HRSA), the Budget provides \$810 million, an increase of \$527 million, for the National Health Service Corps Scholarship and Loan Repayment programs, which support efforts to increase diversity across primary care health professions. According to self-reports by the more than 8,000 Corps clinicians currently providing care – 13.4 percent are African American, 10.1 percent are Hispanic, 6.4 percent are Asian or Pacific Islander, and 1.8 percent are American Indian or Alaska Native. Further, more than half of the nearly 1,100 Corps scholars in the pipeline are minorities – 18 percent are Hispanic, 18 percent are African American, 13 percent are Asian or Pacific Islander, and 2 percent are American Indian or Alaska Native.

HRSA will continue to fund other federally-funded health workforce development programs that are focused on training individuals from disadvantaged and diverse backgrounds in FY 2015. For example, the Centers of Excellence program recruits, trains, and retains underrepresented minority students and faculty with the goal of increasing the supply and quality of underrepresented minorities in the health professions.

The Scholarships for Disadvantaged Students program aims to increase the diversity of the health professions workforce as well as to increase the number of primary care providers working in medically underserved areas while the Nursing Workforce Diversity program works to increase nursing education opportunities for individuals who are from disadvantaged backgrounds including racial and ethnic minorities that are underrepresented among registered nurses. In addition, applicants for health professions training grants must describe their strategies and efforts to produce a health workforce that reflects the diversity of the U.S. population.

The Budget also continues investments in HRSA Health Centers that are essential to ensuring that vulnerable and underserved populations, particularly racial and ethnic minority populations, have access to comprehensive, quality primary care services. Within the requested funding level for Health Centers in FY 2015, HRSA plans to support 150 new access points that will provide care to 900,000 additional patients in high-need communities across the country.

Beyond HRSA, the FY 2015 Budget maintains the FY 2014 doubling of the Minority Fellowship program within the Substance Abuse and Mental Health Services Administration that is part of the President's *Now Is the Time* Initiative for mental health. This funding will provide stipends to graduate students to increase the number of culturally competent behavioral health professionals, including master's level trained behavioral health providers in the fields of psychology, social work, professional counseling, marriage and family therapy, and nursing. In addition, the Budget includes a \$228 million increase for the Indian Health Service to continue its progress in reducing health disparities in Indian Country.

In this tight fiscal environment, the Budget also includes carefully considered reductions and eliminations targeted to activities duplicative of other grant programs within HHS. For example, the Budget proposes to eliminate the Racial and Ethnic Approaches to

Community Health as these activities may be more effectively and efficiently implemented through the new Partnerships in Community Health grant program and State Public Health Actions to Prevent and Control Diabetes, Heart Disease, Obesity and Associated Risk Factors and Promote School Health program, which will provide resources to states to coordinate activities across categorical funding streams.

Language Access and the Health Care Exchange Application Process

An issue of continued concern is the inadequate and often inaccurate translation services provided to limited English proficient Asian language populations – both in translated applications and as it relates to the call-centers established by the Affordable Care Act.

The Secretary mentioned during the March 13, 2014 hearing before this subcommittee that the paper application was not available in 5 languages, and that there are plans to further "diversify the navigator program."

Question 2: In what languages, in addition to English and Spanish, have the paper applications been translated into?

Hard copy paper versions of the application were only available for use in English and Spanish for the first open enrollment period, though the application was translated into many other languages as enrollment aids. These materials can be found at: <http://marketplace.cms.gov/getofficialresources/other-languages/other-languages-materials.html>

Question 3: How does the agency's FY 15 budget request prioritize the translation of paper applications into additional Asian languages, and how does it prioritize in-language services, particularly for Asian language speakers?

The FY 2015 President's Budget requests \$774 million for consumer information and outreach, of which \$703 million will be funded through user fees. This funding will allow for continued support of in-person assistance, such as Navigators, to help individuals apply for coverage. Navigator grantees focus on outreach to specific populations, including those with limited English proficiency. In addition, consumer outreach and education funds development of consumer-focused resources and outreach campaigns. HHS recognizes the importance of targeted outreach messages to underserved communities, such as those with limited English proficiency, is crucial to successfully increasing the number of individuals with insurance coverage.

Essential Community Providers

I was pleased to see the modest improvement to the Qualified Health Plan requirements for Essential Community Providers in the 2015 Letter to Issuers in the Federally-Facilitated Marketplaces. The increase in the fixed percentage standard from twenty to thirty percent is an important first step, but it does not ensure network adequacy for those providers serving the most vulnerable communities.

Question 4: What are the agency's plans for conducting a thorough determination of adequacy to ensure that patients have access to primary care essential community providers – both community-based independent physicians and community health centers – and that ECPs are not being systematically excluded from the networks, or forced into unsustainable reimbursement rates?

In the 2015 Letter to Issuers in the Federally-facilitated Marketplaces, HHS provides that, to be considered to meet the statutory and regulatory Essential Community Providers (ECP) requirements, plans with a network must (1) include in that network at least 30 percent of available ECPs (up from the 20 percent required for 2014) as well as (2) offer contracts in good faith to all available Indian Providers and one ECP from each of 6 ECP categories in each county (if available), one of the six categories is federally qualified health centers. To be offered in good faith, a contract should offer terms that a willing, similarly-situated, non-ECP provider would accept or has accepted. CMS would expect issuers to be able to provide verification of such offers. In addition, any ECPs that the issuer “writes-in” must count towards the denominator when calculating whether a network meets the 30 percent threshold.

Plan networks that do not meet the 30 percent standard must submit for consideration a written explanation of why this was not possible, how the issuer’s provider network(s), as currently designed, provides an adequate level of service for low-income and medically underserved enrollees to meet the statutory and regulatory ECP requirements and how the issuer plans to increase ECP participation in the issuer’s provider network(s) in future years, as necessary.

Issuers may also qualify for an alternate ECP standard to enable them to meet the 30 percent threshold by having an “integrated delivery system,” which provides a majority of covered services through physicians employed by the issuer or through a single contracted medical group. This alternate standard allows issuers to count as ECPs their in-house or contracted providers in health professional shortage areas (HPSA) and other low-income areas in which 30 percent or more of the population falls below 200 percent of the federal poverty level (FPL). Issuers seeking to qualify under the alternate standard but whose networks do not enable them to meet the 30 percent threshold may submit a narrative justification.

Sexual Health Education

We know it is the young people in our country who are bearing the burden of new HIV and other STI infections each year, and that while at a historic low, teen pregnancy in our country is more prevalent than among our global partners.

Question 5: Can you speak to how this Budget addresses the sexual health education needs of young people, particularly those who are typically marginalized (e.g. young black gay men/MSM, Black and Latina young women, youth in foster care, incarcerated youth, etc.), and how this increases prevention?

HHS remains committed to coordinating adolescent sexual health activities to most effectively serve youth throughout the United States. The FY 2015 Budget continues to invest in evidence-based programs that have been shown to reduce teen pregnancy or the risk behaviors associated with teen pregnancy and sexually transmitted infections. Through the Teen Pregnancy Prevention Program (TPP), the HHS Office of Adolescent Health (OAH) supports grants to states, non-profit organizations, school districts, universities, and others to replicate evidence-based programs that have been rigorously evaluated and shown to be effective at reducing teen pregnancies, sexually transmitted infections, or other associated sexual risk behaviors. The TPP program also supports research and demonstration projects that develop and test additional models and innovative strategies to prevent teen pregnancy. HHS also provides funding for state and local education agencies to help districts and schools deliver exemplary sexual health education emphasizing HIV and other STI prevention; increase adolescent access to key sexual health services; and establish safe and supportive environments for students and staff. In addition, the Personal Responsibility and Education Program (PREP), as part of the Affordable Care Act, also funds evidence-based program models through formula grants to states, and tests new strategies through competitive grants to public and private entities. All programs target groups with high teen pregnancy rates.

Questions for the Record **Congressman Mike Honda**

- 1) An estimated 3.5-5.3 million Americans live with chronic viral hepatitis, but between 45%-65% are unaware of their status because of a lack of awareness. Of the \$1.128 billion in the budget request for the CDC's National Center for HIV/AIDS, Viral Hepatitis, STD, and TB Prevention, a mere \$28.7 million dollars is dedicated to viral hepatitis, which must address two very different disease states. As a result, with such little funding apportioned to viral hepatitis, even less is allocated to hepatitis B services. How do you plan on ensuring that funding is dedicated towards viral hepatitis and specifically towards programs in hepatitis B? If you were provided with additional funding to address hepatitis B, what more could your department do?**

Through CDC's implementation of effective vaccination strategies, the annual incidence of hepatitis A virus infection has decreased more than 95% since 1995, and the annual incidence of hepatitis B virus infection, particularly among children aged 15 years and younger, has had a similar decline in the past decade. The 2011 rates of 0.4 new hepatitis A cases and 0.9 new hepatitis B cases per 100,000 members of the nation's population both represent the lowest rates of new cases recorded to date. To achieve the national goal for eliminating mother-to-child transmission of hepatitis B, CDC supports state and local prevention programs to assure that infants exposed to hepatitis B receive recommended prevention services; currently, state and local programs provide services to approximately half of the estimated 24,000 newborns exposed to hepatitis B.

CDC helps its public health partners conduct public health surveillance and investigate outbreaks of hepatitis. CDC detects and responds to outbreaks of hepatitis A and hepatitis B among the large proportion of the U.S. population who remain unvaccinated and vulnerable to these infections. Through these efforts, potentially exposed persons are identified, screened, and referred for treatment if indicated.

In FY 2015, CDC will continue to fund active hepatitis surveillance—investigating case reports to ascertain demographic and infection risk information about patients and disease transmission trends in the community—in a limited number of sites. These jurisdictions will serve as sentinel sites to provide “early warning” viral hepatitis data for the nation as a whole. In addition, CDC will fund prevention research to better inform its programs, focusing on studies of young persons at risk for hepatitis C and studies of ways to improve hepatitis B and hepatitis C screening and linkage to care.

CDC will continue to fund viral hepatitis coordinators in 48 states, the District of Columbia, Los Angeles, New York City, and Philadelphia. Grantees promote the implementation of CDC recommendations for hepatitis vaccination, screening, and linkage to care in the healthcare provider community, raise awareness of the health

disparities caused by viral hepatitis, implement strategies to improve hepatitis A and hepatitis B vaccination, hepatitis B and hepatitis C testing and treatment to reduce morbidity, mortality and transmission caused by these infections. As an example, these coordinators work to incorporate viral hepatitis prevention, testing and linkage to care into appropriate clinical care settings. Additionally, CDC will continue projects begun in 2014 that support the development and evaluation of viral hepatitis prevention programs in a limited number of systems. These viral hepatitis prevention programs will improve the health of communities by reducing new infections, improving systems of care, and combatting hepatitis-related health disparities.

- 2) I have serious concerns about reports of a lack of enrollment materials and assistance for the Asian American and Pacific Islander populations. Limited English proficient Asian Americans and Pacific Islanders can only utilize two of the four ACA enrollment channels: call center and in-person enrollment. Currently, they can neither apply in-language through the on-line portal nor apply in-language through the mail in application option. This is true of any individual who does not speak English or Spanish. There are also reports that the translated materials currently posted on healthcare.gov contain numerous translation and grammatical errors. In addition, we have heard that callers to the language line service experience long wait times, erroneous interpretation, and receive misinformation.**
- a) How does HHS/CMS plan to increase the availability of translated education and enrollment materials in Asian, Pacific Islander and other languages?**
 - b) How are you ensuring that existing materials are of high quality and the information is correct?**
 - c) Who is the language line vendor that you are using and how are you working with the vendor to conduct quality assurance checks?**
 - d) What are your plans for the future of the navigator and certified assistance counselor (CAC) programs to enable in-person assistance for LEP consumers and immigrant families?**

Answer to a and b: CMS is currently planning translation efforts for the second year of open enrollment, and is evaluating the demand and usage of our existing products to help us make decisions about the products we offer given our limited resources. CMS intends to expand the promotion of the availability of translated materials for year two and will continue to make products available through our English and Spanish websites. CMS has quality assurance processes in place to ensure that translations are accurate and high quality.

- c.) Who is the language line vendor that you are using and how are you working with the vendor to conduct quality assurance checks?**

Answer to c: The language line translation service is currently a sub-contract to the primary Call Center contractor.

CMS takes access to translation services very seriously. CMS continuously reviews performance data to ensure that language line services are being offered properly and efficiently to those who need them. Any reports of delayed service or misinformation are researched immediately, and are addressed. To that end, if there are incidents where consumers are reporting service issues, CMS would appreciate specific examples so that they can investigate the call and take corrective action if needed.

d.) What are your plans for the future of the navigator and certified assistance counselor (CAC) programs to enable in-person assistance for LEP consumers and immigrant families?

Answer to d: Training for Navigators and non-Navigator assistance personnel and certified application counselors in the FFM includes training on providing meaningful access to individuals with limited English proficiency (LEP). CMS is currently reviewing this training content for the purpose of making revisions and improvements. FFM-funded Navigators and non-Navigator assistance personnel are permitted to use grant or contract funds to pay for the cost of translation services so that oral translation services can be provided to LEP individuals free of charge. CMS expects certified application counselors to provide appropriate referrals to geographically accessible Navigators, non-Navigator assistance personnel, and/or the Marketplace call center, if the certified application counselor is unable to assist a consumer with limited English proficiency. Certified application counselors are not required to provide oral translation services to the consumers they serve, but are encouraged to do so, if possible. CMS is looking for ways to help certified application counselor organizations be better equipped to provide oral translation services to LEP individuals. For example, they are developing a list of “best practices” related to serving immigrants and LEP individuals, and will be including a presentation on these best practices in an upcoming assister webinar and newsletter.

3. In Silicon Valley, innovation in health technologies is an economic driver. I am dedicated to promoting and supporting innovation in mobile health technologies, which play a significant part of the future of the delivery of health care. What is HHS doing to engage mobile health development? In particular, what is the agency doing to engage mobile health application developers to ensure that their ideas are not only supported but also integrated into the greater mhealth field?

HHS has a number of programs and activities to promote innovation and provide opportunities to integrate solutions into the greater mHealth field. Agencies at HHS including ONC, NIH, CDC, FDA, and ACF fund many mobile health development projects through grants and contracts. ONC leads the HHS effort to encourage a vibrant health IT marketplace. By engaging with developers, delivery systems, providers, patients, and researchers at the leading edge of health IT, ONC is working to find the best

ways to use health IT (including mobile solutions) to meet the goals of better health, improved population health, and greater value.

Examples include:

Innovation Engagement Program: This program facilitates bidirectional learning and mutual understanding between the ONC and the entrepreneurial and developer communities.

Planning 4 Health Innovators Boot Camps: These boot camps are being held nation-wide to educate the health IT startup community about important federal regulations, programs, and opportunities, as well as create a forum for networking and relationship building between federal staff and entrepreneurs.

Code-a-thons: ONC has organized and sponsored 12 weekend-long events in which teams have 24-36 hours to develop solutions to a specified problem. Events are attended by developers, patients, providers, and researchers, and provide a unique opportunity to encourage the spread of ideas and interaction between individuals at different points in the care chain.

Glass Med Challenge: This recent challenge was a public-private partnership with MIT's Hacking Medicine Team and blog MedTech Boston. The challenge was hosted primarily as a lean and virtual effort to crowd source clinical ideas for applications using Google Glass, a new and upcoming wearable technology that has broad applications in health care.

Additionally, ONC has worked collaboratively with FDA and the FCC to develop a draft report that contains a proposed strategy and recommendations on an appropriate, risk-based regulatory framework pertaining to health information technology, including mobile medical applications, that promotes innovation, protects patient safety, and avoids regulatory duplication, in accordance with Section 618 of the Food and Drug Administration Safety and Innovation Act (FDASIA). This framework is relevant to current functionalities and technologies yet sufficiently flexible to accommodate the future and rapid evolution of health IT, including mhealth.

HHS will continue to play an important role to connect technology developers with health/medical experts, policy makers, and scientific researchers to encourage mobile health development.

- 4. Many Asian Americans, Native Hawaiians and Pacific Islanders (AANHPIs) experience barriers in access to mental health services because of language and cultural differences. AAPI's experience high rates of depression, post traumatic stress disorder and thoughts of suicide. Native Hawaiian youth have significantly higher rates of suicide attempts than other adolescents in Hawaii. What is your agency doing**

to address disparities in access to appropriate and culturally competent care for AANHPIs including ensuring that the mental health workforce reflects the diversity of our nation?

HHS is committed to addressing minority health disparities and recognizes that improving and ensuring access to culturally and linguistically appropriate care is a critical step toward achieving behavioral health equity. In addition to HHS' Office of Minority Health's reauthorization in the Affordable Care Act, SAMHSA was directed to establish the Office of Behavioral Health Equity (OBHE). This office has been established and now coordinates agency efforts to reduce behavioral health disparities for diverse populations.

HHS also released the *HHS Action Plan to Reduce Racial and Ethnic Health Disparities* (available at http://minorityhealth.hhs.gov/npa/files/Plans/HHS/HHS_Plan_complete.pdf), which outlines goals and actions HHS will take to reduce health disparities among racial and ethnic minorities. For example, the first priority to "develop disparity impact statements in grantee programs," has resulted in SAMHSA's OBHE developing a disparity impact strategy, built on access to services, services utilized, and outcomes of the services, disaggregated by race and ethnicity among the grantee population served. All grantees are required to submit a disparity impact statement to allow SAMHSA grant programs to identify and track disparities in access, use and outcomes among AANHPI subpopulations in grant service areas; and to implement the National Standards for Culturally and Linguistically Appropriate Services in Health and Health Care as part of a quality improvement plan to address those disparities.

To support the development of a diverse mental health workforce, OBHE manages the National Network to Eliminate Disparities in Behavioral Health (NNED) which brings together, in a virtual network structure, community-based organizations addressing the behavioral health needs of diverse racial, ethnic and LGBT populations. OBHE convenes on-site intensive training for NNED partners, as in April of this year on NNED partner identified high need clinical and organizational practices. Six AANHPI-serving community-based organizations participated in the Achieving Whole Health: A Training for Asian Pacific Islander Wellness Coaches. SAMHSA is also partnering with the Pacific Behavioral Health Collaborating Council (PBHCC) to support the Pacific Behavioral Health Initiative (PBHI) to develop the expertise of a set of Pacific Islander Master Trainer candidates to provide behavioral health training to their Pacific colleagues.

Two other relevant programs SAMSHA supports include the HHS Viral Hepatitis Action Plan and the agency's Minority Fellowship Program. Asian Americans and Pacific Islanders experience high rates of hepatitis. SAMHSA has actively participated in developing and supporting implementation of the HHS Action Plan for the Prevention, Care, & Treatment of Viral Hepatitis. More information on this implementation is available at <http://aids.gov/news-and-events/hepatitis>. As

part of this plan, SAMSHA has promoted integrated care for persons with substance abuse and mental health conditions, including the recent Minority AIDS Initiative Continuum of Care Pilot and the Minority Serving Institutions Partnerships with Community-Based Organizations grant which can be accessed at <http://beta.samhsa.gov/grants/grant-announcements/sp-14-005>. Such programs promote education, prevention and treatment about hepatitis. SAMSHA also has promoted training of Federally Qualified Health Center staff through its Addiction Technology Transfer Centers. The 2012 Viral Hepatitis Action Plan Implementation Progress Report is available at <http://aids.gov/pdf/vhap-2012-interagency-implementation-progress-report.pdf>.

Through the Minority Fellowship Program (MFP), SAMHSA seeks to reduce health disparities and improve health care outcomes of racially and ethnically diverse populations by increasing the number of culturally competent behavioral health professionals available to underserved populations in the public and private nonprofit sectors. The MFP closely aligns with SAMHSA's Eight Strategic Initiatives by addressing the current and projected behavioral health workforce shortages and the need to train providers on recovery-based practices. About 120 MFP Fellows are trained in an average year. The MFP was recently expanded to include behavioral health professionals serving at-risk children and transition-age youth (ages 16-25) and addiction counselors by the President's Now is the Time initiative. Funding opportunity announcements for these expansions are available at <http://beta.samhsa.gov/grants/grant-announcements/sm-14-015> and <http://beta.samhsa.gov/grants/grant-announcements/ti-14-010>.

5. **In your testimony, you noted that minority communities have an increased opportunity for affordable health care coverage. Yet we have not seen disaggregated race or ethnicity data on marketplace and Medicaid enrollment. In my home state, Covered California has been reporting race and ethnicity enrollment data. Why has HHS not released any enrollment data by race or ethnicity? What is your plan to make such information available so we can evaluate the effectiveness of meeting the goal of increasing coverage for minority communities?**

HHS is focused on providing reliable and accurate information related to Marketplace enrollment. We hope to provide additional information in the future, and plan to provide information on race and ethnicity for applicants to the Federally-facilitated Marketplace after the close of open enrollment. We do not require states to report data on race of applicants to us, but note that some states, such as California, have been reporting this information on their own. Reporting of race and ethnicity data from Medicaid will continue through the Medicaid Statistical Information System, and CMS will report this data as soon as it is available.

6. **There are multiple programs within HHS that have a role in the response to the growing problem of antibiotic resistance – including overuse in healthcare, prevention and tracking of resistant pathogens, and development of new antibiotics. In addition to the resources requested for CDC’s antibiotic resistance work, can you describe how Administration plans to tackle antibiotic resistance on an agency-wide basis?**

Efforts to combat antibiotic resistance are supported by the Centers for Disease Control and Prevention (CDC), the National Institutes of Health (NIH), the Food and Drug Administration (FDA), the Assistant Secretary for Preparedness and Response (ASPR), the Centers for Medicare & Medicaid Services (CMS), and the Agency for Healthcare Research & Quality (AHRQ).

To address the threat of antibiotic resistance, CDC supports efforts to better understand the molecular mechanisms of resistance, the development of new clinical diagnostic tests, laboratory activities to assess optimal patient treatment, and the development of strategies to prevent the spread of the emergence of antibiotic resistance across the United States. CDC is also working with regional, state, and local entities to improve surveillance, infection control measures, laboratory diagnostics, and stewardship initiatives. The FY 2015 Budget includes an increase of \$30 million for The Detect and Protect Against Antibiotic Resistance initiative to establish a robust domestic infrastructure that can “detect” antibiotic resistant threats and “protect” patients and communities. This initiative enhances surveillance and laboratory capacity at local, state, and national levels, aiming to characterize domestic antibiotic resistant threats and protect patients from imminent danger. Over five years, CDC has estimated that this initiative can reduce the national incidence of *C. difficile* by 50%, preventing at least 20,000 deaths, 150,000 hospitalizations, and over \$2 billion in healthcare costs.

Within NIH, the National Institute of Allergy and Infectious Diseases (NIAID) is the predominant entity that pursues research to address antibiotic-resistant infections via investments in basic, translational, and clinical research. This research aims to shift the focus of antimicrobial drug development on broad-spectrum therapies that could be used against entire classes of pathogens. NIAID has also committed to support a new leadership group for an antibacterial resistance clinical trial network, which will develop and implement a comprehensive clinical research agenda to address the problem of antibacterial resistance. Within the FY 2015 Budget, NIAID plans to spend approximately \$244 million to continue the support of these and other antimicrobial resistance research activities.

In FY 2015, FDA plans to spend \$29 million agency-wide on activities related to antimicrobial resistance. These resources will fund such activities as reviewing new human antimicrobial products and updating clinical trial guidances to assist industry in the design and conduct of research to support its applications. FDA is also providing recommendations on streamlined development pathways to facilitate the development of new antibacterial drugs for the treatment of patients with unmet medical need. The Center for Drug Evaluation and Research is actively implementing the provisions of the

Generating Antibiotic Incentives Now (GAIN) Act and has granted 45 Qualified Infectious Disease Product (QIDP) designations enabling priority review and fast track status to these products. FDA and NIH plan to hold a public workshop in late July to discuss streamlined antibacterial drug development pathways and common trial designs/master protocols for antibacterial drugs.

The Foods and Veterinary Medicine (FVM) Program continues to move forward to implement Guidance for Industry #213, which would phase out non-therapeutic veterinary uses of antibiotics important to human medicine and to bring therapeutic uses under the supervision of a veterinarian. The FVM Program is also working to support integrated monitoring of antimicrobial resistance among various types of bacteria through the National Antimicrobial Resistance Monitoring System. Other FVM activities include evaluating and ensuring the safety of new antimicrobial products as part of the animal drug approval process, analyzing and summarizing annual sales data for antimicrobial drugs intended for use in food-producing animals, conducting research to enhance methods for characterizing bacteria and to better understand drug effects on bacteria, and developing approaches for collecting data to better understand antimicrobial drug use practices in food-producing animals and the public health impacts of such use.

Within ASPR, the Biomedical Advanced Research and Development Authority (BARDA) supports these efforts through its Broad Spectrum Antimicrobials (BSA) program. The phases of this long-term program include: building and developing a portfolio of antibacterial programs that emphasizes drugs to treat biothreat pathogens and antimicrobial resistant infections; obtaining regulatory approval for hospital or community acquired infection indications; obtaining data sufficient for label expansion for biothreat indications; and building a robust portfolio of unprecedented classes of antibiotics that inhibit novel bacterial targets. In FY 2015, ASPR anticipates devoting a total of \$84 million to support its activities to combat antimicrobial resistance.

In recent years, AHRQ and CMS have supported projects to improve patient safety and reduce healthcare associated infections (HAIs), particularly antimicrobial resistant infections. These projects are specifically concentrated on efforts aimed to reduce the incidence of antibiotic resistant infections, reduce inappropriate antibiotic prescriptions, and better understand the risk factors predisposing patients to drug-resistant infections. For instance, CMS' Medicare Quality Improvement Organizations (QIOs) are boots on the ground educators that work with hospitals to prevent unnecessary catheter use in order to prevent catheter associated urinary tract infections (CAUTIs). Decreased infections means decreased antibiotic prescriptions. In FY 2015, these agencies plan to continue support of these activities.

7. **As a longstanding supporter of chronic disease prevention programs at CDC, I am very interested to learn more about the HHS vision for the new Partnerships for Improving Community Health and other activities at the CDC's National Center for Chronic Disease Prevention and Health Promotion. I was also displeased to see that once again the FY15 HHS budget proposes eliminating the Racial and Ethnic Approaches to Community Health (REACH) program. What is your justification for eliminating the REACH program, especially in light of the recent elimination of the Community Transformation Grant (CTG) program, the only other chronic disease prevention program at CDC that was focusing on the most racial and ethnic disparities in health outcomes in this country? What is your vision for the new Partnerships for Improving Community Health program?**

Partnerships to Improve Community Health (PICH) is a new 3-year initiative to prevent heart disease, stroke, diabetes, obesity, and other leading chronic disease-related causes of death or disability through evidence- and practice-based local-level changes in communities to improve health and health behaviors among the intervention population. The PICH program will support local communities in implementing evidence-based interventions and innovative promising practices to achieve the critical local changes necessary to prevent chronic diseases and their risk factors. The program will mobilize community leadership and resources to bring change to the places and organizations that touch people's lives every day – at work sites, schools, community centers, and health care settings – to reduce the burden of chronic disease. Special emphasis will be directed toward populations that bear a disproportionate burden of disease and lack of access to preventive services, including racial and ethnic minorities.

CDC will fund a mix of urban, rural, and tribal communities to: (1) develop and implement effective models for local action in communities, worksites, schools, and health care; (2) work collaboratively with partners to improve and increase access to quality preventive health services; (3) mentor other communities that want to take action and replicate successful strategies; and (4) support the elimination of racial and ethnic health disparities. In addition, CDC will fund selected national organizations, which will provide support to communities in the form of technical assistance, provision of linkages to resources such as national experts and nontraditional partners, leadership in community evaluation methods, and sustainability planning. All activities supported through this program will contribute to area-wide health improvements and reductions in health disparities.

The FY 2015 budget request eliminates funding for the Racial and Ethnic Approaches to Community Health (REACH) program. These activities may be more effectively and efficiently implemented through the State Public Health Actions to Prevent and Control Diabetes, Heart Disease, Obesity and Associated Risk Factors and Promote School Health program, which will provide resources to states to coordinate activities across categorical funding streams, as well as the Partnerships to Improve

Community Health program and Affordable Care Act Prevention and Public Health Fund investments. For instance, the newly funded Partnerships to Improve Community Health will build on past program successes and lessons learned from CDC's community-based programs. This program will also adopt best practices and lessons learned from the REACH program into its strategy in program planning and implementation.

8. I am concerned the Affordable Care Act Health Benefits Exchanges, including the Federally-Facilitated Exchanges, (FfEs), may not be offering adequate voter registration services, as required by the National Voter Registration Act (NVRA). What is HHS doing to ensure that voter registration as part of the ACA enrollment process?

Section 1413 of the Affordable Care Act requires HHS to develop a single, streamlined application to purchase coverage through the Marketplace and for insurance affordability programs, including premium tax credits, cost-sharing reductions, Medicaid and CHIP. The single, streamlined application contains a link to voter registration materials to assist states and State-based Marketplaces that may use the single, streamlined application with meeting applicable requirements under section 7 of the National Voter Registration Act of 1993 (NVRA). Section 7 of the NVRA requires states to offer voter registration opportunities at certain state and local offices, including public assistance and disability offices. The Department of Justice has determined that State-based Marketplaces, like state Medicaid agencies, must offer voter registration opportunities under the NVRA. Therefore, the paper applications for Marketplace coverage include a link for more information on how applicants can register to vote. When an applicant applies on-line, he or she is also offered a link for more information on how to register to vote. Again, this information is provided to assist states and State-based Marketplaces with meeting applicable requirements under the NVRA.

Funding from the Affordable Care Act to HHS OpDivs 2012-2015 (millions)/ 1

| | FY 2012 | FY 2013 | FY 2014 (estimate) | FY 2015 (estimate) |
|---|---------|---------|-----------------------|-----------------------|
| HRSA | | | | |
| Health Centers | 1,200 | 1,465 | 2,145 | 3,600 |
| School Based Health Centers | 50 | 47 | - | - |
| Family to Family | 5 | - | - | - |
| National Health Service Corps | 285 | 285 | 283 | 310 |
| Home Visiting | 350 | 380 | 371 | - |
| Prevention and Public Health Fund | 37 | 2 | - | - |
| CDC | | | | |
| Early Detection of Certain Medical Conditions Related to Environmental Health Hazards | - | - | - | 20 |
| Prevention and Public Health Fund | 809 | 463 | 831 | 810 |
| SAMHSA | | | | |
| Prevention and Public Health Fund | 92 | 15 | 62 | 58 |
| AHRQ | | | | |
| Prevention and Public Health Fund | 12 | 6 | 7 | - |
| Patient-Centered Outcomes Research Trust Fund | 24 | 58 | 93 | 106 |
| CMS | | | | |
| Affordable Insurance Exchange Grants | 1,655 | 2,148 | 1,319 | 836 |
| Consumer Operated and Oriented Program Contingency Fund | - | 253 | - | - |
| Consumer Operated and Oriented Program (CO-OP) | (400) | (2,279) | - | - |
| Adult Health Quality Measures | 60 | 60 | 60 | - |
| Quality Measurement | 20 | 20 | 20 | - |
| Independence At Home Demonstration | 5 | 5 | 5 | 5 |
| Graduate Nurse Education | 50 | 50 | 50 | 50 |
| Prevention and Public Health Fund | - | 454 | - | - |
| Health Care Fraud Waste and Abuse Program (HCFAC) ACA funding /2 | 65 | 40 | 40 | 30 |
| <i>Offsetting Collections</i> | | | | |
| Risk Adjustment | - | - | - | 3,378 |
| Exchange User Fee | - | - | 200 | 1,159 |
| Risk Adjustment User Fee | - | - | - | 20 |
| Risk Corridors | - | - | - | 5,450 |

| | | | | |
|---|----|----|----|--------|
| Reinsurance Administrative Collections | - | - | - | 20 |
| Transitional Reinsurance | - | - | - | 10,000 |
| ACA Provider Enrollment User Fee | 30 | 25 | 28 | 28 |
| ACF | | | | |
| Health Profession Opportunity Grants | 85 | 81 | 79 | - |
| Personal Responsibility Education | 75 | 71 | 70 | - |
| Abstinence Education | 50 | 47 | 46 | - |
| ACL | | | | |
| Aging and Disability Resource Centers | 10 | 9 | 9 | - |
| Prevention and Public Health Fund | 20 | 9 | 28 | 28 |
| State Health Insurance Programs | 15 | - | - | - |
| Outreach and Assistance for Low-Income Programs | | | | |
| <i>National Center for Benefits Outreach and Enrollment</i> | 5 | - | - | - |
| <i>Aging and Disability Resource Centers</i> | 10 | - | - | - |
| <i>Area Agencies on Aging</i> | 15 | - | - | - |
| OS | | | | |
| Pregnancy Assistance Fund | 25 | 24 | 23 | 25 |
| Prevention and Public Health Fund | 30 | - | - | 105 |
| Patient-Centered Outcomes Research Trust Fund | 6 | 14 | 23 | 26 |

274

- 1/ The FY 2013 and FY 2014 levels reflect levels post-sequestration.
2/ In addition to new funds appropriated to the HCFAC program for CMS displayed in this table, the ACA also included an inflation adjustment to the HCFAC mandatory base funds.

Funding from the Affordable Care Act to HHS OpDivs 2012-2015 (millions)/ 1

| | FY 2012 | FY 2013 | FY 2014 (estimate) | FY 2015 (estimate) |
|---|---------|---------|-----------------------|-----------------------|
| HRSA | | | | |
| Health Centers | 1,200 | 1,465 | 2,145 | 3,600 |
| School Based Health Centers | 50 | 47 | - | - |
| Family to Family | 5 | - | - | - |
| National Health Service Corps | 295 | 285 | 283 | 310 |
| Home Visiting | 350 | 380 | 371 | - |
| Prevention and Public Health Fund | 37 | 2 | - | - |
| CDC | | | | |
| Early Detection of Certain Medical Conditions Related to Environmental Health Hazards | - | - | - | 20 |
| Prevention and Public Health Fund | 809 | 463 | 831 | 810 |
| SAMHSA | | | | |
| Prevention and Public Health Fund | 92 | 15 | 62 | 58 |
| AHRQ | | | | |
| Prevention and Public Health Fund | 12 | 6 | 7 | - |
| Patient-Centered Outcomes Research Trust Fund | 24 | 58 | 93 | 106 |
| CMS | | | | |
| Affordable Insurance Exchange Grants | 1,655 | 2,148 | 1,319 | 836 |
| Consumer Operated and Oriented Program Contingency Fund | - | 253 | - | - |
| Consumer Operated and Oriented Program (CO-OP) | (400) | (2,279) | - | - |
| Adult Health Quality Measures | 60 | 60 | 60 | - |
| Quality Measurement | 20 | 20 | 20 | - |
| Independence At Home Demonstration | 5 | 5 | 5 | 5 |
| Graduate Nurse Education | 50 | 50 | 50 | 50 |
| Prevention and Public Health Fund | - | 454 | - | - |
| Health Care Fraud Waste and Abuse Program (HCFAF) ACA funding /2 | 65 | 40 | 40 | 30 |
| <i>Offsetting Collections</i> | | | | |
| Risk Adjustment | - | - | - | 3,378 |
| Exchange User Fee | - | - | 200 | 1,159 |
| Risk Adjustment User Fee | - | - | - | 20 |
| Risk Corridors | - | - | - | 5,450 |

| | | | | |
|--|----|----|----|--------|
| Reinsurance Administrative Collections | - | - | - | 20 |
| Transitional Reinsurance | - | - | - | 10,000 |
| ACA Provider Enrollment User Fee | 30 | 25 | 28 | 28 |
| ACF | | | | |
| Health Profession Opportunity Grants | 85 | 81 | 79 | - |
| Personal Responsibility Education | 75 | 71 | 70 | - |
| Abstinence Education | 50 | 47 | 46 | - |
| ACL | | | | |
| Aging and Disability Resource Centers | 10 | 9 | 9 | - |
| Prevention and Public Health Fund | 20 | 9 | 28 | 28 |
| State Health Insurance Programs | 15 | - | - | - |
| Outreach and Assistance for Low-Income Programs | | | | |
| National Center for Benefits Outreach and Enrollment | 5 | - | - | - |
| Aging and Disability Resource Centers | 10 | - | - | - |
| Area Agencies on Aging | 15 | - | - | - |
| OS | | | | |
| Pregnancy Assistance Fund | 25 | 24 | 23 | 25 |
| Prevention and Public Health Fund | 30 | - | - | 105 |
| Patient-Centered Outcomes Research Trust Fund | 6 | 14 | 23 | 26 |

1/ The FY 2013 and FY 2014 levels reflect levels post-sequestration.
2/ In addition to new funds appropriated to the HCFAC program for CMS displayed in this table, the ACA also included an inflation adjustment to the HCFAC mandatory base funds.

| BUDS | | 2011 | | 2012 | | 2013 | | 2014 (projected) | | 2015 (projected) | |
|------------------------------------|-------|-------|--------|--------|--------|--------|--------|------------------|--------|------------------|---|
| ACF | | | | | | | | | | | |
| Total FTE used | 1,229 | 1,283 | 1,238 | 1,331 | 1,338 | 1,302 | 1,303 | 1,344 | 1,402 | - | - |
| Total Title 42 FTE | - | - | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 F Staff Count | - | - | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 G Staff Count | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - | - | - |
| AHRO | | | | | | | | | | | |
| Total FTE used | 295 | 297 | 288 | 312 | 313 | 308 | 311 | 326 | 326 | - | - |
| Total Title 42 FTE | 53 | 38 | 35 | 28 | 12 | 14 | 17 | 18 | 18 | - | - |
| Total Title 42 209 F Staff Count | 5 | 3 | 3 | 3 | 6 | 3 | 5 | 4 | 4 | - | - |
| Total Title 42 209 G Staff Count | 48 | 35 | 32 | 25 | 16 | 18 | 33 | 23 | 23 | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - | - | - |
| ACL | | | | | | | | | | | |
| Total FTE used | 112 | 106 | 101 | 100 | 115 | 119 | 146 | 171 | 178 | - | - |
| Total Title 42 FTE | - | - | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 F Staff Count | - | - | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 G Staff Count | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - | - | - |
| CDC | | | | | | | | | | | |
| Total FTE used | 8,569 | 8,944 | 9,567 | 10,179 | 10,674 | 10,877 | 11,134 | 11,134 | 11,134 | - | - |
| Total Title 42 FTE | 702 | 796 | 922 | 991 | 1,100 | 1,225 | 1,315 | 1,315 | 1,313 | - | - |
| Total Title 42 209 F Staff Count | 94 | 97 | 105 | 104 | 127 | 138 | 125 | 124 | 124 | - | - |
| Total Title 42 209 G Staff Count | 747 | 879 | 1,009 | 995 | 1,002 | 686 | 664 | 837 | 837 | - | - |
| Total # Title 42 Recruitment Bonus | 5 | 5 | 14 | 5 | 11 | 2 | 2 | 5 | 5 | - | - |
| Total # Title 42 Retention Bonus | 8 | 5 | 3 | 4 | 4 | 2 | 4 | 4 | 4 | - | - |
| CMS | | | | | | | | | | | |
| Total FTE used | 4,526 | 4,483 | 4,381 | 4,537 | 5,195 | 5,416 | 5,889 | 6,044 | 6,380 | - | - |
| Total Title 42 FTE | 15 | 13 | 9 | 8 | 6 | - | - | - | - | - | - |
| Total Title 42 209 F Staff Count | 1 | 1 | 1 | 1 | 1 | - | - | - | - | - | - |
| Total Title 42 209 G Staff Count | - | 0 | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - | - | - |
| FDA | | | | | | | | | | | |
| Total FTE used | 9,618 | 9,909 | 11,310 | 12,522 | 13,331 | 13,538 | 14,141 | 15,872 | 16,905 | - | - |
| Total Title 42 FTE | 533 | 551 | 795 | 870 | 858 | 15,432 | 842 | 851 | 851 | - | - |
| Total Title 42 209 F Staff Count | 251 | 249 | 232 | 223 | 215 | 940 | 914 | 573 | 573 | - | - |
| Total Title 42 209 G Staff Count | 394 | 406 | 698 | 800 | 795 | 864 | 771 | 808 | 808 | - | - |

| | | | | | | | | | |
|------------------------------------|--------|--------|--------|--------|--------|--------|--------|--------|--------|
| Total # Title 42 Recruitment Bonus | 1 | 65 | 55 | 5 | 0 | 3 | 0 | 2 | 2 |
| Total # Title 42 Retention Bonus | 114 | 95 | 120 | 18 | 26 | 26 | 17 | 22 | 22 |
| HRSA | | | | | | | | | |
| Total FTE used | 1,708 | 1,484 | 1,481 | 1,609 | 1,869 | 1,894 | 1,902 | 1,940 | 1,983 |
| Total Title 42 FTE | 3 | 3 | 4 | 4 | 4 | 6 | 7 | 6 | 6 |
| Total Title 42 209 F Staff Count | - | - | - | - | 3 | 4 | 14 | 7 | 7 |
| Total Title 42 209 G Staff Count | - | - | - | - | - | 2 | 7 | 4 | 4 |
| Total # Title 42 Recruitment Bonus | - | - | - | - | 1 | - | - | 1 | 1 |
| Total # Title 42 Retention Bonus | - | - | - | - | 1 | 1 | 1 | 1 | 1 |
| IHS | | | | | | | | | |
| Total FTE used | 15,062 | 14,985 | 15,267 | 15,830 | 15,509 | 15,432 | 15,393 | 15,610 | 15,760 |
| Total Title 42 FTE | 2 | 2 | 1 | 4 | 3 | - | - | - | - |
| Total Title 42 209 F Staff Count | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 G Staff Count | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - |
| NIH | | | | | | | | | |
| Total FTE used | 16,986 | 17,241 | 17,785 | 18,351 | 18,569 | 18,497 | 18,234 | 18,234 | 18,234 |
| Total Title 42 FTE | 4,234 | 4,348 | 4,537 | 4,766 | 4,792 | 4,771 | 4,554 | 4,639 | 4,639 |
| Total Title 42 209 F Staff Count | 2,334 | 2,458 | 2,530 | 2,580 | 2,533 | 2,565 | 2,273 | 2,488 | 2,488 |
| Total Title 42 209 G Staff Count | 2,432 | 2,541 | 2,609 | 2,729 | 2,834 | 2,859 | 2,797 | 2,810 | 2,810 |
| Total # Title 42 Recruitment Bonus | 59 | 72 | 62 | 53 | 32 | 12 | 11 | 27 | 27 |
| Total # Title 42 Retention Bonus | 75 | 68 | 55 | 26 | 16 | 15 | 17 | 19 | 19 |
| OS | | | | | | | | | |
| Total FTE used | 3,850 | 4,069 | 4,118 | 4,706 | 5,416 | 5,456 | 5,297 | 5,432 | 5,888 |
| Total Title 42 FTE | 10 | 11 | 11 | 11 | 12 | 14 | 13 | 13 | 13 |
| Total Title 42 209 F Staff Count | 5 | 2 | 7 | 8 | 10 | 12 | 11 | 10 | 10 |
| Total Title 42 209 G Staff Count | 3 | 4 | 1 | 5 | 7 | 8 | 4 | 6 | 6 |
| Total # Title 42 Recruitment Bonus | - | - | - | 1 | 1 | - | - | 1 | 1 |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - |
| PSC | | | | | | | | | |
| Total FTE used | 1,126 | 1,132 | 1,199 | 985 | 826 | 764 | 634 | 695 | 695 |
| Total Title 42 FTE | 1 | - | - | - | - | - | - | - | - |
| Total Title 42 209 F Staff Count | - | - | - | - | - | - | - | - | - |
| Total Title 42 209 G Staff Count | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Recruitment Bonus | - | - | - | - | - | - | - | - | - |
| Total # Title 42 Retention Bonus | - | - | - | - | - | - | - | - | - |
| SAMHSA | | | | | | | | | |
| Total FTE used | 511 | 533 | 519 | 530 | 542 | 590 | 608 | 655 | 655 |
| Total Title 42 FTE | 10 | 11 | 7 | 6 | 0 | 2 | 1 | 1 | 1 |
| Total Title 42 209 F Staff Count | - | - | - | - | - | 1 | 1 | 1 | 1 |

| | | | | | | | | | |
|---|--|--|--|--|--|--|--|--|--|
| Total Title 42 209 G Staff Count | | | | | | | | | |
| Total # Title 42 Recruitment Bonus | | | | | | | | | |
| Total # Title 42 Retention Bonus | | | | | | | | | |
| Annual Estimated Title 42 Recruitment and Retention Bonus | | | | | | | | | |
| Grand Total Recruitment and Retention Bonus | | | | | | | | | |
| ACF | | | | | | | | | |
| AHRQ | | | | | | | | | |
| ACL | | | | | | | | | |
| CDC | | | | | | | | | |
| CMS | | | | | | | | | |
| FDA | | | | | | | | | |
| HRSA | | | | | | | | | |
| IHS | | | | | | | | | |
| NIH | | | | | | | | | |
| OS | | | | | | | | | |
| PSC | | | | | | | | | |
| SAMHSA | | | | | | | | | |
| Annual Estimated Title 42 Recruitment and Retention Bonus | | | | | | | | | |
| Grand Total Recruitment and Retention Bonus | | | | | | | | | |
| ACF | | | | | | | | | |
| AHRQ | | | | | | | | | |
| ACL | | | | | | | | | |
| CDC | | | | | | | | | |
| CMS | | | | | | | | | |
| FDA | | | | | | | | | |
| HRSA | | | | | | | | | |
| IHS | | | | | | | | | |
| NIH | | | | | | | | | |
| OS | | | | | | | | | |
| PSC | | | | | | | | | |
| SAMHSA | | | | | | | | | |
| Annual Salary Cap | | | | | | | | | |
| ACF, AHRQ, Aca, CMS, HRSA, IHS, OS, PSC, SAMHSA | | | | | | | | | |
| CDC, FDA, NIH | | | | | | | | | |

*The cap can be exceeded, only, with the approval of the HHS Secretary

| Annual Maximum Refundable Bonus | | | | | | | | | | | |
|---------------------------------|----------|----------|----------|----------|----------|--------------|--------------|------|------|------|---------------------|
| | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 (through 2018) |
| ACF | - | - | - | - | - | - | - | - | - | - | - |
| AHRQ | - | - | - | - | - | - | - | - | - | - | - |
| ACL | - | - | - | - | - | - | - | - | - | - | - |
| CDC | \$67,500 | \$35,089 | \$37,400 | \$53,750 | \$53,359 | \$ 10,728.00 | \$ 50,571.00 | - | - | - | - |
| CMS | - | - | - | - | - | - | - | - | - | - | - |
| FDA | \$19,800 | \$33,750 | \$20,492 | \$40,000 | \$0 | \$ 75,000.00 | \$0.00 | - | - | - | - |
| HRSA | - | - | - | - | \$22,375 | - | - | - | - | - | - |
| IHS | - | - | - | - | - | - | - | - | - | - | - |
| NIH | \$1,250 | \$57,250 | \$50,000 | 45,000 | \$62,500 | \$ 85,000.00 | \$ 46,250.00 | - | - | - | - |
| OS | - | - | 0 | \$20,097 | \$25,000 | - | \$0.00 | - | - | - | - |
| PSC | - | - | - | - | - | - | - | - | - | - | - |
| SAMHSA | - | - | - | - | - | - | - | - | - | - | - |

| Annual Maximum Vacation Bonus | | | | | | | | | | | |
|-------------------------------|----------|----------|----------|----------|----------|--------|--------|------|------|------|---------------------|
| | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 (through 2018) |
| ACF | - | - | - | - | - | - | - | - | - | - | - |
| AHRQ | - | - | - | - | - | - | - | - | - | - | - |
| ACL | - | - | - | - | - | - | - | - | - | - | - |
| CDC | \$46,602 | \$38,196 | \$25,877 | \$20,973 | \$25,367 | 22,776 | 38,496 | - | - | - | - |
| CMS | - | - | - | - | - | - | - | - | - | - | - |
| FDA | \$32,608 | \$17,980 | \$26,492 | \$14,606 | \$13,630 | 5,459 | 6,400 | - | - | - | - |
| HRSA | - | - | - | - | \$4,975 | 44,600 | 11,150 | - | - | - | - |
| IHS | - | - | - | - | - | - | - | - | - | - | - |
| NIH | \$49,831 | \$68,999 | \$44,681 | \$45,001 | \$45,845 | 45,842 | 45,845 | - | - | - | - |
| OS | - | - | - | - | - | - | - | - | - | - | - |
| PSC | - | - | - | - | - | - | - | - | - | - | - |
| SAMHSA | - | - | - | - | - | - | - | - | - | - | - |

| Annual Number of Title 42 Hires | | | | | | | | | | | |
|---------------------------------|------|------|------|------|------|------|------|------|------|------|---------------------|
| | 2007 | 2008 | 2009 | 2010 | 2011 | 2012 | 2013 | 2014 | 2015 | 2016 | 2017 (through 2018) |
| ACF | - | - | - | - | - | - | - | - | - | - | - |
| AHRQ | - | - | - | - | - | - | - | - | - | - | - |
| ACL | 5 | 2 | 3 | 4 | 1 | 6 | 20 | - | - | - | - |
| CDC | - | - | - | - | - | - | - | - | - | - | - |
| CMS | 169 | 192 | 183 | 198 | 179 | 143 | 106 | - | - | - | - |
| FDA | - | - | - | - | - | - | - | - | - | - | - |
| HRSA | 197 | 178 | 322 | 165 | 168 | 479 | 379 | - | - | - | - |
| | - | - | - | 4 | 2 | 1 | 1 | - | - | - | - |

WEDNESDAY, MARCH 26, 2014.

**BUDGET HEARING—FUTURE OF BIOMEDICAL
RESEARCH**

WITNESSES

**FRANCIS S. COLLINS, M.D., PH.D., DIRECTOR, NATIONAL INSTITUTES
OF HEALTH, DEPARTMENT OF HEALTH AND HUMAN SERVICES**

HAROLD E. VARMUS, M.D., DIRECTOR, NATIONAL CANCER INSTITUTE

**ANTHONY S. FAUCI, M.D., DIRECTOR, NATIONAL INSTITUTE OF AL-
LERGY AND INFECTIOUS DISEASES**

**STORY C. LANDIS, PH.D., DIRECTOR, NATIONAL INSTITUTE OF NEURO-
LOGICAL DISORDERS AND STROKE**

**GARY H. GIBBONS, M.D., DIRECTOR, NATIONAL HEART, LUNG, AND
BLOOD INSTITUTE**

Mr. KINGSTON. Well, the hearing will come to order, and I welcome the NIH team today and welcome all the committee members to this hearing on biomedical research and the public health budget. And, Dr. Francis Collins, you know that this committee supports biomedical research. We support the NIH and the good work that you are doing.

I kind of have two ongoing discussions with you, as you know it. Number one, I do not think we really tell the story as well as we could about what NIH has done, and I always wish that you would just come in here with a table full of displays and assortments of things that you have invented and lives you have improved.

You know, I have said many times that I wish all of America knew about the 1 percent reduction in cancer rate in the last several years and how that saves \$500 billion a year to the taxpayers. I do not think we brag enough about what has happened with AIDS in Africa in terms of the reduction of that. And I do not think we brag enough about polio, and I know that is partly NIH and CDC and a lot of other efforts. But there are so many things that have gone on that I think we need to brag about it more.

And the other part, which is maybe the yin and the yang, you might say, is the pushback that we get particularly from the conservative side of public side in terms of how some of those grants end up with questionable grantees. And you may remember last year Dr. Harris brought up a \$7 million grant to a group in California that wanted to make a determination of if a Tea Party was receiving tobacco money or not. And while somebody might want to know that, I want the money going into the cure for cancer.

You know, and in some of our discussions you have said, well, you know, 33,000 grants, we cannot control all of them. But, you know, I would say also when we are spending tax dollars, if I went over to the Library of Congress and just tore up one of the books, one of the millions of books that are over there, it would still be

egregious. And so, I think we have to have zero tolerance when it comes to frivolous grants.

There are some other examples, and I will just list them, and we have had discussions about this. But the influence of personal responsibility rhetoric on public health, the impact of New York City's sugar sweetener beverage policies on calories purchased and consumed, a randomized trial of internet access to nicotine patches, research ethics education in the Balkans and Black Sea countries, capturing the content of adolescent Facebook communications, experimental design of a social security system in the Yucatan, cigarette smoke detecting underwear, public health education campaigns in China. And so, you know, to me the question is not completely on the merits of this research because I can understand how some people somewhere would want to know about the social security system in the Yucatan. But the reality is it does not have to do with biomedical research.

And so, what we need to do as good stewards of taxpayers' money is what I have said so many times to you publically and privately, and I think you and I are on the same page, is I want the money to go to the scientists with the white jacket in the lab finding the cure to the next world-changing cure, you know, disease or whatever that we can be ahead of. And I have also said to you part of it is helping me help you. And when we get the pushback on these other things, then it hurts our ability to help you.

The other thing that I wanted to mention is when people come into our offices, which they do all year long, and they are absolutely always welcome. But so often they know about your budget, but they do not know about the \$800 million tap, which this Administration takes out of the NIH. After we have appropriated the money, this Administration assesses you \$800 million and actually wanted to assess you more.

And the thing about these grants is if you do a grant to a group that we do not necessarily like or maybe we do like, maybe we embrace, maybe we denounce. But it is transparent. The tap money that goes back to Health and Human Services, it is not transparent, and we do not know what happens to that money. Not that that is your responsibility at all, but I do wish that in the advocacy family of NIH, people would realize that the tap does take \$800 million out of money that we appropriated to you. So I wanted to say that.

Ms. DeLauro, I want to yield to you and Ms. Lowey. Well, let me go ahead and yield to you, and then I will introduce the panel, if that is okay. All right? Thanks.

Ms. DELAURO. Thank you very much, Mr. Chairman. I would just say one comment before I make my statement, actually two very short comments. I think with your comment with the regard to the grant and the lobbying connection, I think it is important to note that the OIG, the Office of the Inspector General, at HHS said in response to the letter that you wrote, and they wrote back on September 9th, "In our review of the records for these four grant awards, we found no evidence that the grants violated prohibitions on the use of Federal funds for lobbying, publicity, or propaganda." I think that ought to be part of the record.

Ms. DELAURO. And sometimes with regard to research, I know when I sat on the Ag Committee—I still sit on the Ag Committee, but I was both ranking member and I was chair of that committee. When someone said to me we were going to do research on the glass-eyed sharpshooter, I said and what could that be research on? Well, if you talk to any of the vintners all over the Nation, the glassy-eyed sharpshooter destroys vines and grapes, et cetera, which destroys an industry. So sometimes just to the eye of the observer, it is not always what it appears to be. And though it would be good to express a view on what some of that research is about and how it comports with the kinds of research that is important.

Let me just say thanks, Mr. Chairman, and good morning. This is thrilling, it really is, and I think I may have said this in the past—Dr. Collins, director, Dr. Varmus, Fauci, Landis, Gibbons—and to talk about the 2015 budget for the NIH. I am a Yankees fan, and so I will transpose this phrase. It used to be when it was DiMaggio and the rest of the folks, it was Murderers Row, but I would say that this is a savior's row that we have here this morning. So thank you so much for your insights, for your expertise, for all that you do every single day in saving lives. And you push the frontiers of medical science.

You all do know that I am a cancer survivor. It is 28 years actually this month, March, cancer free, and I am here by the grace of God and biomedical research and the work that you do. So I am in awe of what you do. The work supported by NIH saved my life as it has saved countless other lives. So as we discuss the issues today, I hope the subcommittee will not just look at the budgetary costs of NIH programs, but also the huge costs to our health, our society, and our economy, and even to knowledge itself if we fail to invest in health research and disease prevention.

The simple fact is the scientific and medical breakthroughs supported by NIH have allowed millions of Americans, myself included, to live happier and healthier lives. Because of this life-saving research, we have seen dramatic reductions, as the chairman pointed out, in heart disease, stroke fatalities. The 5-year survival rate for childhood leukemia has risen to 90 percent. Fewer than 50 babies are born with HIV a year in America. We now have a cervical cancer vaccine.

NIH has given us all of this while growing our economy at the same time. Every dollar that goes to NIH grants results in \$2.21 of local economic growth. It is over a 100 percent return on the investment. Discoveries arising from NIH funded research are the foundation for our entire biomedical industry. That vital sector exports an estimated \$90 billion in goods and services annually. It employs one million U.S. citizens with wages totaling an estimated \$84 billion.

Just consider the economic benefit of one NIH supported research initiative. Our \$4 billion investment in the human Genome Project spurred an estimated \$796 billion in economic growth from 2000 to 2010. It is a 141-fold return on our investment. I cannot say enough to you, Dr. Collins. Congratulations on the triumph. And I do not want to even think of what we would be doing and where we would be without the Genome Project.

Given the priceless value of better health and longer lives for so many Americans, as well as the amazing rates of return, ensuring that the NIH is adequately funded should be a fundamental priority for this subcommittee. It is why we came together in a bipartisan way to double the NIH budget 15 years ago. And yet recent budget policies have been shrinking NIH. Its total funding is now \$700 million less than it was before sequestration, \$1.2 billion than it was just 4 years ago. Only 58 percent of the deep sequester cuts were restored in the 2014 budget.

When adjusted for increasing costs of medical research, NIH has lost more than 19 percent of its purchasing power since 2005, and here again, NIH is being forced to do less with less. And if our allocation is not increased, it will be much harder to do right by the NIH and all of our other priorities moving forward. The cuts have a direct, devastating impact on innovative medical research that saves lives, that boosts our economy.

NIH estimates that it will be able to support over 2,000 fewer research project grants in 2014 than it did in 2012, and over 5,000 fewer grants than in 2004. Ten years ago, NIH was able to fund almost 1 out of every 3 applications for research grants. Now that "success rate" is down to less than 1 in 5. Understand the loss. Cutting medical research is an incalculable loss: the discovery of fundamental knowledge about how we grow, how we age, how we become ill. And that may be dramatically slowed down, and so, too, may new treatments for the prevention and treatment of disease.

Biomedical research gives us the gift of life. That is what the NIH represents. I hope and I trust that we will keep that in mind as we consider how we move forward today. Thank you very, very much, Mr. Chairman. And particularly thank you to all of our witnesses this morning.

Mr. KINGSTON. No passion.

[Laughter.]

Mr. KINGSTON. Ms. Lowey.

Ms. LOWEY. Thank you, Mr. Chairman. Thank you, Madam Ranking Member. We have been sitting together on this panel for a very long time, and I often say to my good friend I feel a little guilty. You are coming here to answer our questions and respond when you really should be doing so much more important work in your individual laboratories, your offices. And we thank you for your brilliance and your contribution. Thank you for being here.

I must say there is no agency that I am prouder to support than the National Institutes of Health. My top priority in this subcommittee is to increase investments in biomedical research. In fact, I saw you the other night, Dr. Fauci, at Research America, and John Porter spoke. And we remember when he took the lead on the other side of the aisle. We worked together to double investments in biomedical research.

This has never been a Democrat or Republican issue. We have all worked together. Maybe we will do it this year, Mr. Chairman, again, and then you can be honored by Research America. It was a wonderful, wonderful evening. Years ago we did this. It was, as I mentioned, bipartisan, and it substantially increased our investments. Not only does the NIH's work lead to future improvements

in quality of life, it is also an economic engine with every dollar generating 2.2 in economic activity.

And I recall, Mr. Chairman, those days before we had a budget, before we were able to work together. And I spoke to some people in some of the labs in our major institutions that are doing research funded by the National Institutes of Health. And they were saying those labs are really at a standstill, and a lot of the best and the brightest kids were not signing on. They went off to Google probably or someplace else in the private sector because they did not know when the next grants were coming, and this is really key. And I always remind all my friends that investments in the NIH is an economic engine as well with every dollar generating \$2.21 in economic activity.

So if we want to remain a global leader, we must make research and development a priority. Germany's federal investment in health R&D has increased by 60 percent since 2005. A number of others are accelerating investments. This is consistent with overall research and development spending. In the last 10 years, U.S. expenditures as a share of economic output have remained nearly constant while China's has increased nearly 90 percent, South Korea's nearly 50 percent. We cannot afford flat budgets that hamper innovation.

Mr. Chairman, I had an interesting chat with one of the scientists in my district. It was a small laboratory, three of them, and they went over to China just to see what was going on. They were offered a laboratory—I do not know if they are as brilliant as any of ours—with 45 scientists, a whole equipped lab. And this is what China is doing to try to increase their investments in their NIH.

So, Mr. Chairman, we cannot afford flat budgets. I am in awe of the brilliant leaders here today, and I thank you so much for having this hearing. I yield back.

Mr. KINGSTON. Thank you, Ms. Lowey, Ms. DeLauro. Do any other members wish to have an opening statement?

[No response.]

Mr. KINGSTON. If not, I will go ahead and introduce our very distinguished panel. Dr. Harold Varmus, who is no stranger to the Hill. He is now the director of the National Cancer Institute, and a Nobel laureate, and the former director of the NIH. And I guess we are skipping around—Dr. Landis—I'm not sure why. Dr. Fauci, National Institute of Allergy and Infectious Disease, we are glad to have you here. And back to you, Dr. Story Landis, director of the National Institute of Neurological Disorders and Stroke, and Dr. Gary Gibbons, director of the National Heart, Lung, and Blood Institute. And, of course, Dr. Collins, Francis Collins, the Director of NIH.

We stick with the 5-minute rule here, and so whether you are speaking or whether committee members, the clock is blind, and so we are going to adhere to that. However, Ms. DeLauro and I find that sometimes spilling over that, there are some advantages of it. And I would like to ask unanimous consent to just, because I have already seen your testimony, and I have to go, as I had explained to you earlier, to two other hearings. But if I could ask the committee for unanimous consent to cross examine the witness before. I just want to have a question. Are we okay with that?

HUMAN GENOME DEMONSTRATION

Do you have the human genome demonstration in your pocket that you sometimes carry?

Dr. COLLINS. I do.

Mr. KINGSTON. I did not see that in your testimony. I am extremely happy that you brought that. And maybe before I leave, you can explain—that is not in your testimony.

Dr. COLLINS. It is not because there is so much that I could say.

Mr. KINGSTON. Okay. Well then, this is my unanimous request to the committee. Could you explain that because that is the kind of see and tell thing members of the public need to know about. And so, before I leave, I would like you—and this will not count against your 5 minutes.

Dr. COLLINS. Would you prefer that I do that first or give the statement?

Mr. KINGSTON. I would love to hear it now because I am very excited about it, and I am sure Rosa and Ms. Lowey have seen it, but I do not know if the rest of you all have seen this. And he is not going to say this, but he was actually the one in charge of the project that mapped the human genome.

Dr. COLLINS. And, Mr. Chairman, that was an amazing ride, I can tell you, to have the opportunity to oversee an effort, which when it was first started in 1990, many people thought was a little bit too ambitious, and maybe not something that could be achieved in a 15-year period. But we did it. In fact, we got that project done ahead of schedule and under budget, and I am glad to say with Federal funds involved. And you already heard the way in which this has paid off economically with 141-fold return on that investment in terms of what has happened since 2003.

But one of the things that has happened is this continued amazing set of advances in the technology, much of which has been stimulated by a grants program through the Genome Institute at NIH to try to encourage people to come up with ever-faster, cheaper, better ways to do DNA sequencing. And that has gotten us in the space of this 10-year period from the cost of a human genome running at about \$400 million now to this year an announcement by one of the companies that is making these machines built upon NIH technology that they can do it for \$1,000. And that has been sort of this mythical goal that you would get to the \$1,000 genome, and 2014 seems to be the time where that has happened.

This particular gadget I am holding is a DNA sequencing machine that uses very micro-scale technology, as you can imagine, and allows you to sequence a complete human genome in the space of about 2 and a half days. This fits into a larger machine that actually does the read out, but this is it. You put the DNA sample in. You add re-agents to these ports, the chemical, and enzymatic reactions are carried out. The letters of the DNA code, A, C, G, and T, are read out, and there you have it.

Imagining going from sequencing machines the size of a phone booth or even bigger to this is, I think, a wonderful testimony to American ingenuity and to the opportunity for the Federal government to be really entrepreneurial to make these kinds of things

happen in partnership with the private sector and all that they can do to make those dreams happen.

So thank you for asking about it. It is a great story to be able to tell.

Mr. KINGSTON. Thank you, and I yield back. And you may begin your testimony.

SUMMARY STATEMENT OF DR. FRANCIS COLLINS

Dr. COLLINS. All right. Thank you. Well, good morning again, Chairman Kingston, Ranking Member DeLauro, members of the subcommittee. And I am not going to read my written testimony, but a shortened version of it.

It is a great honor to appear before you. This panel has a long history of supporting NIH's mission to seek fundamental knowledge and apply it in ways that enhance human health, lengthen life, and reduce suffering. NIH and millions of patients are truly grateful for your leadership.

First, my colleagues and I would like to thank you for the recent Fiscal Year '14 Omnibus Appropriation for NIH. This subcommittee came together in a bipartisan way to reverse the downward spiral of support for NIH that has cost us almost 25 percent of our purchasing power for research over the last 10 years. While difficult trade-offs made it impossible to reverse completely the devastating effects of that sequester, we are gratified it was at least possible to turn the corner this year.

Thank you also for holding this hearing today. Indeed, the future of biomedical research, the title of this hearing, has never been brighter. And my colleagues and I look forward to discussing a few of the many opportunities that lie ahead.

In recent years, we have made tremendous strides in our understanding of human disease. Basic science has led the way. Advances in genomics, proteomics imaging, and other technologies have led to the discovery of more than a thousand new risk factors for disease and biological changes that may serve as future therapeutic targets.

But still, we must do more than aim to just understand disease. We must find new ways to treat and prevent it. As just one example, NIH-funded scientists are well on their way to developing a universal influenza vaccine. Such a vaccine would not only eliminate the need for an annual 'flu shot, but would also provide protection against outbreaks, like the H5N1 and H7N9 events in Southeast Asia that are causing considerable concern right now.

Another major challenge is exploring what has been called biology's final frontier, the most complicated structure in the known universe, the human brain with its 86 billion neurons. As you know, NIH is leading the brain research through advancing innovative neurotechnologies—that is an acronym, BRAIN Initiative—and we are grateful for your support. This initiative will provide a foundational platform for major advances in Alzheimer's disease, autism, schizophrenia, traumatic brain injury, epilepsy, and many other brain disorders.

A third area of scientific opportunity I want to highlight today involves one of our Nation's biggest killers, cancer. Until recently, our weapons for attacking cancer have been largely limited to sur-

gery, radiation, and chemotherapy, all of which can be effective, but carry risks. Recent advances have given us insights into the intricate workings of the cancer cell using genomic tools, for instance, and a whole new generation of targeted therapeutics is emerging, ushering in an era of individualized precision medicine.

Furthermore, and this is very hot stuff, we figured out a way to harness the body's own immune system to fight this dreaded disease. In one of those new approaches, certain types of immune cells, called T-cells, are collected from cancer patients and engineered to produce special proteins on their surface. When these engineered T-cells are infused back into patients, they have the power to seek and destroy cancer cells.

Now, knowing how to turn T-cells into little Ninja warriors required big investments in basic biomedical research over more than a decade. But promising results in both adults and children with leukemia led Science magazine to name cancer immunotherapy as the 2013 breakthrough of the year, not just for the U.S., for the whole world; not just for biomedical research, but for all of science.

One patient who volunteered for this experimental therapy is Doug Olson of Pipersville, Pennsylvania. Doug was diagnosed with chronic lymphocytic leukemia at the age of 49. After four rounds of chemotherapy over more than a decade failed to induce remission, his only option was a bone marrow transplant, a risky procedure with a 50 percent success rate. But then Doug heard about a clinical trial of cancer immunotherapy at the University of Pennsylvania, and he signed up. The therapy was administered. Within days several pounds of cancer cells had melted away. Three weeks later researchers could detect no sign of leukemia in his blood. Today, more than 3 years later, they still cannot find it.

From the standpoint of both the scientific and human perspectives, that is truly amazing. And Doug is back to living life to the fullest. If you look over here you will see that he is even running a half marathon with his son. That is Doug in the white tee shirt on the right.

[The information follows:]

"A lot of people get
to have hope now.
The world has
been transformed
a little bit, and I got
to be a part of it."



I believe there are many more stories like Doug's on the horizon. Our Nation has never witnessed a time of greater promise for advances in medicine. With your support, we can realize our vision for accelerating discovery across the vast landscape of biomedical research from basic scientific inquiry to human clinical trials.

The National Institutes of Health is ready to move forward, so thank you for your support of NIH. My colleagues and I welcome your questions.

[The statement of Francis S. Collins, M.D., Ph.D. follows:]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

The Future of Biomedical Research

Witness appearing before the

House Subcommittee on Labor – HHS – Education Appropriations

Francis S. Collins, M.D., Ph.D.

Director, National Institutes of Health

March 26, 2014

Good morning, Mr. Chairman and distinguished Members of the Subcommittee. I am Francis S. Collins, M.D., Ph.D., Director of the National Institutes of Health (NIH). It is an honor to appear before you today to provide an overview of NIH's critical role in enhancing our nation's health through scientific discovery.

As the nation's biomedical research agency, NIH's mission is to seek fundamental knowledge about the nature and behavior of living systems and to apply that knowledge to enhance human health, lengthen life, and reduce illness and disability. I can report to you that NIH leadership, employees, and grantees continue to believe passionately in this mission.

Before I discuss the tremendous strides we have made and the exciting scientific opportunities on the horizon, I want to thank you, Mr. Chairman, and Ranking Member DeLauro, as well as your colleagues, for the recent Fiscal Year (FY) 2014 Omnibus Appropriation bill. The Subcommittee came together in a bipartisan way to increase funding for NIH and we are truly grateful for your action. The past year has been challenging for us: the sequester reduced funding for groundbreaking medical research and affected the morale of the scientific community. This impact was further exacerbated by the shutdown.

There is much good news to report about the science that we support. NIH has been advancing our understanding of health and disease for more than a century; scientific and technological breakthroughs generated by NIH-supported research are behind much of the gains our country has enjoyed in health and longevity. For example, deaths from heart attack have fallen by more than 60 percent over the past 40 years, while deaths from stroke have declined 70 percent. Cancer death rates have been dropping about 1 percent annually for the past 15 years—life expectancy gains that save the nation billions of dollars. HIV/AIDS treatment and prevention now enable us to envision the first AIDS-free generation since this virus emerged

more than 30 years ago. NIH research also has given us vaccines to protect against an array of life-threatening diseases, including cervical cancer, influenza, and meningitis. We can look forward to a future in which advanced prevention and treatment strategies such as these allow everyone to have a significantly better chance of living a long and healthy life.

These statistics tell you how far we have come—but our aim is to go even further, faster. Let me describe a few of the many areas in which NIH-supported research is opening up extraordinary opportunities to improve the health of the American public.

A major program that began this year is the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative, for which thanks are due to this Subcommittee for its FY 2014 support. NIH is a major player in this pioneering multi-agency venture that will enable the creation of new tools capable of examining the activity of billions of nerve cells, networks, and pathways in real time. By measuring activity at the scale of circuits and networks in living organisms, we can begin to decode sensory experience and, potentially, even memory, emotion, and thought. Successful pursuit of the BRAIN Initiative will revolutionize neuroscience, providing a foundational platform for major advances in Alzheimer's disease, autism, schizophrenia, epilepsy, traumatic brain injury, and many other brain disorders.

As technology allows us to tackle mind-boggling tasks like recording the activity of billions of nerve cells in the brain or determining the DNA sequence of tens of thousands of human genomes, researchers are generating enormous quantities of data at an unprecedented pace. The challenge posed by this revolution is how to store, retrieve, integrate, and analyze this mountain of complex data—and transform it into knowledge that can improve human health. To address this challenge that affects virtually all areas of biomedical research, we have just launched the Big Data to Knowledge (BD2K) initiative. The goals of BD2K are to develop and

disseminate new analytical methods and software, enhance training of data scientists, and facilitate broad use and sharing of complex biomedical datasets. With sustained investment and effort, we will overcome the challenges associated with Big Data to accelerate real-world applications of basic science discoveries.

We are also excited about another area of intense interest: the development of therapeutics. Recent advances in genomics, proteomics, imaging, and other technologies have led to the recent discovery of more than a thousand risk factors for disease—biological insights that ought to hold promise as targets for drugs. But drug development is a terribly difficult and failure-prone business. To the dismay of researchers, drug companies, and patients, the vast majority of drugs entering the development pipeline fall by the wayside. The most distressing failures occur when a drug is found to be ineffective in the later stages of development—in Phase II or Phase III clinical studies—after years of work and millions of dollars have already been spent. A major reason for such failures is that scientists often have not had enough information to choose the right biological targets. If a drug is aimed at the wrong target, it won't work against the disease it was intended to treat.

With that challenge in mind, we were thrilled last month to launch the Accelerating Medicines Partnership (AMP). This unprecedented public-private effort will use cutting-edge scientific approaches to sift through a very long list of potential therapeutic targets, and choose those most likely to lead to success. Besides NIH, the AMP partners include the FDA, 10 biopharmaceutical firms and a number of non-profits, including patient advocacy groups. This pre-competitive partnership, which will share all data openly, will initially focus on three disease areas that are ripe for discovery: Alzheimer's disease, type 2 diabetes, and the autoimmune

disorders, lupus and rheumatoid arthritis. Through this team effort, we believe we can reach our shared goals of treating and curing disease faster.

Preventing disease is another top priority, and influenza is one area of prevention in which we are poised for rapid progress. Currently, to provide protection against the rapidly evolving influenza virus, a new vaccine must be produced each year and we all need to get an annual flu shot. Also, despite best efforts, the vaccine isn't always ideal. In an average year, the flu claims up to 49,000 American lives and costs the U.S. economy about \$87 billion. But it does not have to be that way. NIH-funded researchers are now working on a universal flu vaccine—designed to protect people against virtually all strains of the flu for extended periods of time and, thus, potentially reduce the need for annual flu shots. Of critical importance, such a vaccine could also protect against a future global flu pandemic.

While we are several years away from having a universal flu vaccine available to the public, our researchers have already demonstrated proof of concept and are testing a number of approaches, including two-stage “prime boost” vaccines and ferritin nanoparticles. Clearly, the prospect of a universal flu vaccine is not science fiction. Early clinical studies are already underway. With sustained investment, the United States may be a few years away from realizing its potential to benefit our health and our economy.

As impressive as a universal flu vaccine would be, it is not the only trick we are teaching our immune systems. We are also aiming to harness the body's own immune system to fight cancer. Until recently, our weapons for attacking cancer have been largely limited to surgery, radiation, and chemotherapy—treatments that carry risks and cause adverse side effects. Now, after years of intense basic and translational research, we have an exciting new possibility: cancer immunotherapy.

Researchers have long been puzzled by the uncanny ability of cancer cells to evade the immune response. What stops the body from waging its own “war on cancer?” As it turns out, our bodies have built-in checkpoints to prevent our immune systems from going into overdrive and killing healthy cells. Now, NIH-funded researchers have discovered a way to genetically modify certain white blood cells called T-cells—the soldiers of the immune system—to attack tumor cells. In this new approach, T-cells are collected from cancer patients and engineered in the lab to produce special proteins on their surface, called chimeric antigen receptors (CARs). When the modified cells are infused back into patients, they multiply and, with guidance from their newly engineered receptors, seek and destroy tumor cells. Promising results in patients with leukemia prompted *Science* magazine to name this its 2013 Breakthrough of the Year.

Today, I have provided a very brief overview of NIH’s past successes and continuing commitment to basic, translational, and clinical research. Our nation has never witnessed a time of greater promise for advances in medicine. With your support, we can anticipate a future of accelerating discovery across NIH’s broad research landscape, from fundamental scientific inquiry to human clinical trials. The “National Institutes of Hope” is ready to move forward.

This concludes my testimony, Mr. Chairman. I look forward to your questions.

PREPARED STATEMENT OF HAROLD E. VARMUS, M.D

Mr. Chairman and Members of the Committee:

I am pleased to present the President's budget request for the National Cancer Institute (NCI) of the National Institutes of Health (NIH). The Fiscal Year (FY) 2015 NCI budget of \$4,930,715,000 includes an increase of \$7,944,000, or 0.2 percent, compared to the FY 2014 level of \$4,922,771,000.

Overview of NCI Research Priorities

This is an era of remarkable opportunity in cancer research. Armed with broad knowledge about how various cancers arise and with powerful new research tools, the NCI is well equipped to accelerate progress towards preventing, diagnosing, and treating cancer more effectively. This era of opportunity is due in significant part to the subcommittee's consistent support for biomedical research at NCI and NIH.

The resources that you provide allow NCI to address an ambitious challenge: reducing the incidence, morbidity, and mortality for all of the many types of cancer, with tangible benefits for all Americans. The FY 2015 budget will allow the NCI to build on the tremendous progress in many areas of cancer research, with the aim of improving outcomes for patients with all types of cancer.

I will summarize some recent accomplishments and highlight new opportunities in five areas of NCI-supported research – genomics, cancer immunology, targeted therapeutics, bioinformatics, and prevention – to illustrate the breadth and pace of NCI's progress.

The **Cancer Genomics** research that NCI supports has dramatically altered our understanding of how cancer develops, identified the molecular signatures that can be

used to diagnose and categorize cancer more precisely, and provided new targets for therapeutic intervention. For example, two major initiatives – TCGA (The Cancer Genome Atlas) and TARGET (Therapeutically Applicable Research to Generate Effective Treatments) – have addressed nearly twenty common adult cancers and several less common cancers that occur in adults and children, revealing both tissue-specific patterns of genetic changes and changes that are common to several types of cancers. TCGA is a joint initiative of the NCI and the Human Genome Research Institute. During the past year, TCGA published comprehensive characterizations of acute myeloid leukemia, endometrial cancer, and clear cell renal carcinoma, among others. While every cancer is distinct genetically, many changes in the genome are shared among a wide array of cancer types, and each type of cancer has distinct patterns that often reflect exposure to carcinogenic agents, such as tobacco smoke and ultraviolet radiation. As these massive surveys come to conclusion, the NCI's Center for Cancer Genomics is leading efforts to make full use of the TCGA results, including the best ways to incorporate genomic findings into the design of clinical trials.

Some of the surprising findings from the TCGA and TARGET projects – such as the involvement of genes that govern the chemistry of chromosomal proteins, that influence cell metabolism, and that guide the processing of RNAs and proteins – are influencing the study of cancer biology throughout the NCI's programs. TCGA and TARGET will certainly enlarge our understanding of carcinogenesis and will likely open new frontiers for preventing, diagnosing, and treating cancers.

Cancer immunology is a rapidly advancing field that, in just the past few years,

has dramatically altered our understanding of host defenses in response to cancers. It has also produced new and well-validated methods for treating cancer using antibodies that attach to proteins on cancer cell surfaces and using methods that modulate the complex behavior of the immune system to attack cancer cells.

For several years, monoclonal antibodies against cancer cell proteins have been used to treat blood cancers, such as certain lymphomas and leukemias, and subsets of several types of solid tumors, such as breast and colorectal cancer. More recently, immunotoxins have been created by genetic engineering to fuse antibodies with parts of bacterial toxins to selectively kill cancer cells. For example, such immunotoxins developed in the NCI intramural program have induced remissions in late stage cases of mesothelioma, ovarian cancer, triple-negative breast cancer, drug-resistant hairy cell leukemia, and childhood acute lymphoblastic leukemia.

There is also great optimism within the science community about modulating the immune system by introducing novel antigen receptors into cancer-killing T cells and especially by infusing antibodies that interfere with a system that impedes the immune response to cancer cells. These “immune-modulating” antibodies have recently received FDA approval, and other antibodies that bond other immune cell regulators may soon follow. In 2011, FDA approved a monoclonal antibody, called ipilimumab, to treat advanced melanoma. Some patients with metastatic melanoma being treated with ipilimumab are still alive several years after completing treatment. In 2013, another promising antibody to treat melanoma – lambrolizumab – received “breakthrough” designation by the FDA, helping expedite its development and further

use in clinical trials, with the possibility of an expedited FDA review. In recognition of these and other recent achievements in the field of immunology, and the promise of further developments, “cancer immunotherapy” was named this year’s Breakthrough of the Year by *Science* magazine.

Targeted therapies, based on the use of drugs that inhibit specific proteins implicated in the behavior of cancer cells, are now being developed and tested for their effects in patients with many types of cancer. Over the past decade, FDA has approved several drugs that rely on this therapeutic approach to treat cancers of blood cells, lung cancer, melanoma, and other cancers, and many more are in development. This activity has accelerated because of discoveries in genomics, cell signaling pathways, chemistry, and structural biology, and with the identification of new ways to inhibit proteins that are required for the integrity of cancer cells.

Mutant RAS proteins are perhaps the most prominent potential targets for new therapies that the academic and commercial research sectors have thus far failed to target with inhibitory drugs. The importance of the RAS gene family in cancer has been clear for over thirty years; one family member, K-RAS, is mutated in more than 90 percent of pancreatic adenocarcinomas, about 40 percent of colorectal cancers, and about 25 percent of lung adenocarcinomas. For this reason, the NCI recently launched the RAS Project, a large-scale collaboration between investigators at the NCI’s Frederick National Laboratory for Cancer Research and those in NCI’s intramural and extramural communities. The RAS Project is motivated in part by new developments in the study of RAS proteins, including new information about their structural properties,

binding of mutant RAS proteins to mutant-specific inhibitors, interactions with other cellular proteins required for function, and new tests for genes required to allow RAS mutants to exert their effects.

Still, while pursuing a path that leads to “precision medicine,” the NCI must also maintain its capacity to test new ways to deploy the currently dominant means of therapy. For instance, a recent study of patients with metastatic prostate cancer showed markedly increased survival in men who received chemotherapy when starting anti-androgenic hormone therapy, a result that is likely to change clinical practice for a cancer that continues to kill about 30,000 American men annually.

Drug resistance commonly emerges in cancers being treated with either traditional chemotherapies or novel targeted therapies, allowing disease to progress. Over the past decade, NCI-supported studies have revealed several mechanisms by which resistance occurs, including additional mutations affecting the target molecules, mutations in related genes, and changes in gene expression. In some cases, especially chronic myeloid leukemias, drugs that overcome resistance have been identified, developed and FDA-approved. But in other situations, resistance to targeted drugs remains a major impediment to success, and the NCI is making major investments to study this problem.

Bioinformatics, the management of enormous sets of molecular and clinical data is a critical component of NCI’s toolkit to study cancer in all of its manifestations. In work that ranges from cancer genomics, to cell signaling, and to clinical trials, the proper collection, analysis, storage, retrieval, and distribution of “big data” are critical

elements of the Institute's charge. The NCI's Center for Bioinformatics and Information Technology (CBIIT) is addressing these responsibilities, in conjunction with NCI divisions. Part of the current effort requires the costly development of "cloud computing" to work with the vast (petabyte) amounts of genomic data generated by TCGA, TARGET, and other projects, and to assemble and ultimately integrate clinical data with genomic data in manageable forms to promote further discovery and improve cancer care.

Prevention of cancer remains NCI's most desired goal. While complete avoidance of cancer may be impossible, since cancers often arise through spontaneous mutations, the control of tobacco use, vaccination against cancer-causing viruses (human hepatitis B virus and human papillomaviruses), sunlight avoidance, and regulation of dietary and carcinogenic substances (such as asbestos) have already reduced the incidence and the mortality rates of many cancers. For instance, between 2001 and 2010, largely due to the earlier reductions in tobacco use, there was a 25 percent decrease in male death rates and an eight percent decrease in female death rates due to lung cancer, the major cause of death from cancer in the United States. Likewise, vaccination with current HPV vaccines can drastically reduce the incidence and mortality of several types of cancer, including cervical, anal, and oropharyngeal cancers that are caused by infection with certain strains of HPV.

Still, NCI recognizes that these successes are incomplete, and therefore invests heavily in efforts to address several pertinent behavioral and biological questions. For instance, despite dramatic declines in the use of tobacco, about 18 percent of Americans continue to smoke. New approaches are needed to convince young people not to use

tobacco and to convince current smokers to quit. Use of HPV vaccines remains far from the desired levels among adolescent girls and boys in the United States, as the February 2014 report from the President's Cancer Panel emphasized. Better methods to promote the use of these potentially lifesaving vaccines are needed, at the same time as the dosing schedules and the protective breadth of the vaccines are improved.

Conclusion

An important measure of the overall success of NCI's work is the annual "Report to the Nation," which describes trends in the incidence and death rates in the United States for many types of cancer. As has now been true for over a decade, the most reliable indicator – death rates from all cancers combined for men, women, and children – continues to decline by about one and a half percent per year. This reduction represents the savings of an enormous number of years of life and can be ascribed in large measure to the work of the NCI to prevent and treat cancers more effectively.

Still, although mortality rates have been decreasing for most cancers, progress has not occurred as rapidly as desired, and for some cancers the numbers have not improved – or have worsened. Thus, much work remains. But the overall success apparent from both the public health data and recent achievements in the laboratory and clinical sciences inspires the NCI's conviction that expanded efforts on all frontiers of cancer research will produce better health in the United States and around the globe.

PREPARED STATEMENT OF GARY H. GIBBONS, M.D.

Mr. Chairman and distinguished members of the Subcommittee:

I am pleased to present the President's Budget request for the National Heart, Lung, and Blood Institute (NHLBI) of the National Institutes of Health (NIH). The fiscal year (FY) 2015 budget of \$2,987,685,000 includes an increase of \$4,948,000 over the FY 2014 enacted level of \$2,982,737,000.

NHLBI's highest priorities for research investment are conditions that contribute substantially to the global burden of disease. Heart and lung diseases are the leading causes of death, disability, and rising health care costs from non-communicable diseases in the United States and worldwide. Research supported by the NHLBI has contributed to dramatic improvements in longevity, quality of life, and the wealth of the nation. Deaths from cardiovascular disease, for example, have dropped by 70 percent in the past 40 years. This success reflects a balanced approach to supporting discovery science that spans basic, clinical, and population research. As accountable stewards seeking to maximize the public's return-on-investment, we are committed to continually improving our approach to strategic priority-setting and systematic evaluation of our portfolio to ensure the highest possible impact on science and health.

Reflecting upon the NHLBI's legacy of success, many of the previous advances involved interventions at the latter stages of chronic disease. The FY 2015 budget envisions a research agenda that elucidates the underlying mechanisms of disease such that clinicians can more accurately predict at-risk individuals and tailor preventive interventions for disease long before symptoms and irreversible damage occur. Our strategic vision is guided by the breathtaking scientific opportunities at hand and public

health needs, in consultation with domain-experts at the leading edge of discovery science. The FY 2015 budget continues a journey toward predictive, preventive precision medicine that holds promise for turning research-to-results, continuing the dramatic decline in the burden of chronic disease in our nation.

UNPRECEDENTED SCIENTIFIC OPPORTUNITIES

Sustained investments in fundamental discovery science have led to new tools and technologies that stand to revolutionize medical research and clinical practice. Biomedical advances in congenital heart disease (CHD), the most common structural birth defect, have led to dramatic improvements in infant survival over the past 50 years, now with more adults living with CHD than children. However, current palliative approaches that repair birth defects have limitations that compromise the length and quality of life. Recent NHLBI-supported research, applying the latest genomic technologies, has identified spontaneous genetic mutations that increase the risk of CHD. This breakthrough finding is beginning to unlock the mysteries of CHD, helping to define what goes awry during the formation of the heart and lay the foundation for preventing or fixing defects in the womb. To that end, NHLBI is investing in regenerative medicine research to enhance the capacity of the heart to repair itself. The 2012 Nobel Laureate, Shinya Yamanaka, is part of a large inter-institutional team of NHLBI-funded investigators studying how to use a child's own cells to repair a congenital defect or create a tissue graft that could grow as a child ages.

NHLBI investments in reparative biology and tissue bioengineering may also hold promise for accelerating new drug development platforms in partnership with the private sector. For example, NHLBI-funded investigators at Stanford University are

using stem cells derived from adult tissue in a laboratory to create heart cells and model diseases such as those that perturb the electrical system of the heart in atrial fibrillation. These models are being used to more efficiently screen many novel drugs to determine efficacy as well as potential toxicities, augmenting the discovery pipeline.

PREEMPTING AND PREVENTING CHRONIC DISEASE

New scientific discoveries hold promise for making public health inroads to halt chronic diseases before they become debilitating. In sickle cell disease (SCD), for example, we have made great strides in reducing complications from the disease, such as penicillin to prevent fatal infections in infants, transfusions to reduce stroke risk, and hydroxyurea to reduce pain and hospital admissions. While these advances have extended lifespans from childhood into the sixth decade of life, they target complications not the disease itself—a disease that disproportionately affects African Americans (about 1 in 500 births). We recently funded a new program that we hope will lead to the next generation of SCD treatments. Particularly exciting are studies that are attempting to raise fetal hemoglobin levels (the most powerful known modifier of SCD severity) through modulation of a gene called *Bcl11A* that is involved in the switch from fetal to adult hemoglobin during development. These studies open the door to potential treatments that can reactivate the fetal hemoglobin gene to inhibit the sickle cell shape change of red blood cells, which could preempt disease progression.

Chronic obstructive pulmonary disease (COPD), the third leading cause of death, is a prime example of a chronic disease in which biomedical research advances have ameliorated symptoms; yet most interventions fail to dramatically alter the natural course of the disease. There is a critical need to identify at-risk individuals earlier in

the disease process to prevent disease progression. NHLBI's COPDgene study is integrating genetics and imaging studies to characterize pre-clinical subtypes of COPD. Such characterization can enable clinicians to detect subtle changes in lung function and structure long before symptoms develop, conventional clinical tests show abnormalities, or progressive lung damage occurs. This leading-edge research points to a horizon of individualized, precision medicine to preempt chronic lung disease.

TRANSLATING DISCOVERIES INTO PUBLIC HEALTH IMPACT

While basic science is the cornerstone of scientific discovery, it is the beginning of a long path to public health impact. NHLBI has been a leader in traversing this road. Noted research initiatives like the Framingham Heart Study first identified the cardiovascular disease risk factors now addressed in routine physicals, which led to basic research that won Brown and Goldstein the Nobel Prize for their research on cholesterol metabolism—setting the stage for the development of statin drugs.

We are currently amidst the unfolding of a similar story. The recent discovery of a mutation in the gene PCSK9 among a family with very low LDL cholesterol levels and reduced risk of heart attack has led to basic science discoveries and the rapid development of PCSK9 inhibitors. This public-private partnership is moving toward potential widespread clinical use as the next generation of cholesterol lowering drugs.

We now know, however, that we must look beyond one-size-fits-all treatments. Population science and genetics research have clearly demonstrated individual differences not only in predisposition to disease but also in treatment response. For example, 26 million Americans currently suffer from asthma—the leading cause of missed school days for children and a driver of preventable hospitalizations and

emergency room visits. Asthma disproportionately affects African Americans; African American children are twice as likely to have asthma as white children and, as adults, are two to three times more likely to die of asthma than any other racial or ethnic group. While effective treatments exist, they do not reach all of those in need. NHLBI will be seeking applications focused on identifying barriers and testing strategies to enhance the implementation of evidence-based practices in diverse communities across the nation. Beyond the current treatments, next generation therapies should target these differences to achieve maximal benefit. NHLBI's multi-center clinical trial network, AsthmaNet, is beginning the Best African American Response to Asthma Drugs (BARD) study to compare the effectiveness of different treatments on the management of asthma in African Americans. BARD will also assess how genetics may influence an individual's response to the treatments, which could be a paradigm shift in addressing challenges like disparities in asthma care.

CONCLUSION

We are in the midst of a very exciting period in science in which the capacity to enhance human health has never been greater. New tools and technologies are daring us to envision a future that is unburdened by chronic heart, lung, and blood diseases—not only ensuring wellness but also increasing economic productivity and reducing health care costs. For example, research shows that treating patients at moderate risk for cardiovascular disease with statin drugs to lower cholesterol can reduce annual medical spending by up to \$430 million. Imagine how much can be saved by preventive interventions earlier in the disease course before symptoms begin and the

costs of treatment rise dramatically. By achieving that goal, the return-on-investment of biomedical research will strengthen both the health and the wealth of the nation.

PREPARED STATEMENT OF ANTHONY S. FAUCI, M.D.

Mr. Chairman and Members of the Committee:

I am pleased to discuss current and future plans for biomedical research at the National Institute of Allergy and Infectious Diseases (NIAID) of the National Institutes of Health (NIH). The President's Fiscal Year (FY) 2015 NIAID budget request of \$4,423,357,000 is approximately \$31 million more than the FY 2014 funding level (\$4,392,670,000).

NIAID conducts, supports, and translates basic and clinical research into the development of diagnostics, therapeutics, and vaccines to detect, treat, and prevent infectious and immune-mediated diseases. NIAID has a dual mandate that balances research addressing current biomedical challenges with the capacity to rapidly respond to new threats from emerging and re-emerging infectious diseases and bioterrorism.

INFECTIOUS DISEASES RESEARCH

HIV/AIDS. NIAID is leading transformational progress in basic and clinical research on HIV/AIDS. The decades-long NIAID investment in HIV/AIDS research has made the goal of an AIDS-free generation a possibility with sustained effort. NIAID continues to improve and refine HIV prevention and treatment tools, including antiretroviral therapies to effectively manage disease and reduce HIV transmission, and pre-exposure prophylaxis to protect against HIV. NIAID also is advancing research toward the development of an effective HIV vaccine to complement existing prevention strategies. HIV vaccine development will be informed by NIAID efforts to identify immunological markers in the subset of people protected against HIV infection in the RV144 trial, the first HIV vaccine trial to show modest efficacy. The NIAID Vaccine

Research Center together with several NIAID grantees are making rapid progress on ways to generate broadly neutralizing antibodies to protect against multiple strains of HIV, research that may translate to vaccines and therapeutics of global public health significance.

Years of NIAID-supported research on HIV pathogenesis and the role of HIV reservoirs have suggested the feasibility of curing some HIV-infected individuals. NIAID will investigate promising reports of a handful of infants who were born HIV-positive but now test negative for the virus following aggressive antiretroviral treatment initiated shortly after birth by supporting a clinical trial to determine if this strategy is safe and effective for other infants. NIAID also will play a major role in implementing the President's \$100 million HIV/AIDS cure research initiative. As part of this effort, NIAID will support additional research on HIV latency and persistence. Understanding these processes may reveal new strategies toward a cure.

NIAID recently restructured its HIV/AIDS Clinical Trials Networks to capitalize on the growing body of promising HIV research findings and to better address current research questions. The Networks will focus on improved ways to prevent and treat HIV, tuberculosis and hepatitis C co-infections, and on research toward development of a vaccine, microbicides, and a cure.

Tuberculosis. Tuberculosis (TB) remains a significant cause of illness and death throughout the world, especially among those also infected with HIV. NIAID recently launched a genome sequencing project that will examine the genetic diversity of TB bacteria and patterns of drug resistance to understand TB pathogenesis and to identify new drug targets and molecular mechanisms of resistance. This research will be

particularly important to address the emergence of multi- and extensively drug-resistant TB. NIAID-supported scientists also are working to modify the existing antibiotic spectinomycin to bypass mechanisms of resistance to this drug. These efforts have shown promise in TB animal models.

Malaria. NIAID continues to progress toward its goal to control, eliminate, and ultimately eradicate malaria worldwide. The development of vaccines is a critical part of this endeavor. NIAID researchers and grantees recently completed an early-stage clinical trial that showed a novel vaccine composed of weakened malaria sporozoites was safe and protected against malaria. NIAID has developed two new tests to rapidly and inexpensively detect resistance to artemisinin, a first-line antimalarial drug. NIAID also is exploring innovative methods to control the spread of malaria. For example, NIAID-funded researchers have established a bacterial infection that passes from female mosquitoes to their offspring and kills malaria parasites within the mosquitoes before they can infect humans.

Other Infectious Diseases of Domestic and Global Health Importance. NIAID is committed to research on infectious diseases affecting global health. Influenza is among the most important infectious diseases of domestic and global concern. NIAID research addresses the challenge of seasonal influenza and prepares for the threat of an emerging pandemic. NIAID is developing and evaluating vaccines against the avian influenza strains H5N1 and H7N9 to deploy if needed to prevent further spread among humans. NIAID also is examining these vaccines paired with adjuvants – components that enhance the immune response – to provide the greatest protection with the smallest dose possible. NIAID investigators and grantees are making significant progress toward the

development of a universal influenza vaccine that could generate durable protection over a period of years against a wide range of seasonal and pandemic influenza strains. Studies conducted by NIAID scientists at the NIAID Special Clinical Studies Unit in the NIH Clinical Center are providing important clues into the susceptibility and immune response of patients to influenza infection. Future studies will examine the effectiveness of new vaccines and therapeutics.

Respiratory syncytial virus (RSV) is a serious respiratory infection primarily of young children that causes significant illness and hospitalizations in the U.S. and thousands of deaths worldwide. There is no vaccine to protect infants and children against RSV. Researchers at the NIAID Vaccine Research Center recently determined the structure of a key RSV protein bound to a broadly neutralizing human RSV antibody and used it to design an experimental RSV vaccine that is effective in animal models. NIAID has advanced this groundbreaking RSV vaccine into early-stage clinical trials in humans. *Science* magazine highlighted this discovery among the top 10 scientific breakthroughs in 2013.

Hepatitis C virus (HCV) is a significant cause of chronic liver disease and cancer, and often co-infects people with HIV. Traditional HCV therapies frequently have severe side effects and may not be successful in many patients. NIAID and NIH Clinical Center investigators recently led a Phase II trial of a new HCV drug, sofosbuvir. The trial demonstrated that sofosbuvir, combined with the antiviral drug ribavirin, was highly effective and well tolerated even in patients predicted to have poor outcomes with traditional HCV treatments. Sofosbuvir and similar therapies for the

treatment of HCV have recently been approved, potentially revolutionizing treatment outcomes.

Antimicrobial resistance is a significant public health challenge and an NIAID priority. NIAID recently reassessed research needs for this important issue and established a Leadership Group to design, implement, and manage the clinical research agenda for a new antibacterial resistance research network. NIAID provides resources to lower the investment risk for industry, academia, and non-profit organizations to facilitate a robust pipeline of diagnostics, vaccines, and therapeutics for resistant microbes.

RESEARCH ON IMMUNOLOGY AND IMMUNE-MEDIATED DISORDERS

NIAID's commitment to research on basic and clinical immunology continues to foster important insights that ultimately will help to better treat and prevent immune-mediated disorders, including food allergy. NIAID-funded investigators recently demonstrated that female sex hormones affect the gut microbiome and promote development of autoimmunity in an animal model, providing clues into why women are more likely to be affected by autoimmune diseases. NIAID-supported researchers have made progress in understanding how exposure to certain microbes in early life, especially those found in homes with dogs, may protect against the development of asthma and other allergies. NIAID grantees also developed two urine tests to diagnose and predict rejection of a transplanted kidney. These simple tests could one day replace the invasive procedure currently used to detect organ rejection and particularly would benefit African Americans, who are disproportionately affected by organ transplant rejection.

CONCLUSION

For more than 60 years, basic and clinical research conducted and supported by NIAID on infectious and immune-mediated diseases has spurred the development of vaccines, therapeutics, and diagnostics to improve the health of millions around the world. NIAID will continue to perform the basic, clinical, and translational research critical to advancing the health of our nation and the world.

PREPARED STATEMENT OF STORY C. LANDIS, PH.D.

Mr. Chairman and Members of the Committee: I am pleased to present the President's Budget request for the National Institute of Neurological Disorders and Stroke (NINDS) of the National Institutes of Health (NIH). The Fiscal Year (FY) 2015 NINDS budget of \$1,608,461,000 includes an increase of \$22,664,000 over the comparable FY 2014 level of \$1,585,797,000. NINDS supports research to reduce the burden of neurological disorders, from basic studies of the normal brain through clinical trials of prevention and treatment interventions. Today, I will make four points: 1) the burden of neurological disorders is enormous; 2) past NINDS research has paid off; 3) opportunities for future progress are extraordinary; and 4) we have well informed plans to exploit these opportunities.

BURDEN OF NEUROLOGICAL DISORDERS

Nearly 800,000 Americans experience a stroke each year, and 15 to 30 percent of the 6.8 million stroke survivors alive today suffer permanent disability.¹ Traumatic brain injury (TBI) is the leading cause of death and disability in children and young adults, common among the elderly, and a major concern for the military and veterans. In the United States, 2.5 million people receive emergency care for a TBI each year, and millions more suffer mild TBI (concussions). Epilepsy affects 2.3 million Americans, including 1 in 26 people at some time in their lives. Alzheimer's disease is receiving increasing attention, but most people are less aware that frontotemporal dementia (FTD) is the most common dementia in people under age 60, and vascular

¹ Statistics for stroke, TBI, and epilepsy from U.S. Centers for Disease Control and Prevention www.cdc.gov

dementia, which affects blood vessels in the brain, is the second most common dementia overall and is so closely intertwined with Alzheimer's disease that most dementia patients have a combination of the two. Parkinson's disease, spinal cord injury, cerebral palsy, multiple sclerosis, and hundreds of rare diseases that affect children and adults add to the immeasurable human and economic burden.

PROGRESS FOR PATIENTS AND FAMILIES

NINDS research drives progress directly, and indirectly catalyzes private sector advances. NINDS studies on risk factors and prevention contributed to a decline in the age-adjusted stroke death rate by 35.8 percent from 2000 to 2010; the actual number of stroke deaths fell 22.8 percent.² NINDS research developed the only approved emergency drug therapy that restores blood flow to the brain following stroke, increasing likelihood of recovery with little or no disability by 30 percent. Research has also demonstrated, defying conventional wisdom, a wider window of opportunity for stroke rehabilitation—even patients who start rehabilitation as late as six months after a stroke can improve, and patients can continue to improve one year after a stroke. For people with epilepsy, an implantable device approved this year senses impending seizures and delivers electrical pulses to stop them. Long-term NINDS research provided the essential foundation for private sector development of this device. Similarly, NINDS research directly and indirectly contributed to deep brain stimulation (DBS) therapies now in use for Parkinson's, essential tremor, and dystonia and under clinical testing for many other disorders, as well as to development of drugs for multiple sclerosis—10 are now on the market, including the first oral drugs. Overall,

² Circulation 2014; 129:e28-e292

the private sector has nearly 450 medicines in development for neurological disorders, which would not be possible without the foundation of NIH research³.

EXTRAORDINARY OPPORTUNITIES

Science and technology are opening unprecedented opportunities for progress against neurological disorders. Studies on the normal brain build the foundation. Notable recent advances, for example, revealed how the brain clears out debris during sleep, how molecular structures called ion channels control electrical activity, and the first human “connectome” maps, providing astonishing views of the basic wiring diagram of living, thinking human brains. Advances in stem cell biology now enable researchers to reproduce in cell culture key steps in amyotrophic lateral sclerosis (ALS) and other disorders using brain cells derived from patients’ own skin cells. Basic science has led to new insights that explain how chronic pain is wired in the brain, what happens in the brain following a concussion, and how cell-to-cell propagation of abnormally folded proteins could drive progression of Parkinson’s, Alzheimer’s, and other neurodegenerative disorders. New gene sequencing methods and high throughput gene silencing technologies have accelerated the discovery of genes that cause epilepsy and revealed potential new drug targets for Parkinson’s disease. In a few dramatic cases, gene discoveries have led directly to treatments that help patients with rare disorders, including subtypes of dystonia and childhood neurodegenerative disease, but more often painstaking translational research is required to advance genetic and other discoveries toward therapies. Among the many examples, promising reports in

³ 2013 Report: Medicines in Development for Neurological Disorders, Pharmaceutical Researchers and Manufacturers of America <http://www.phrma.org/innovation/meds-in-development>

laboratory animals this year demonstrated a drug therapy that prevented the development of epilepsy, cell transplants that controlled seizures, natural growth factor rescue of neonatal brain injury, therapies that improved cognition in Down syndrome, and a hand neuroprosthesis that restored touch sensation as well as movement.

PROGRAMS AND PRIORITIES

NINDS relies heavily upon the wisdom and ingenuity of researchers throughout the United States to propose and evaluate the best scientific opportunities.

Complementing investigator-initiated programs, NINDS initiatives target unmet opportunities or public health needs. Institute priorities reflect strategic and disease-specific planning that engages the scientific community and the public, and rigorous evaluation of programs, closing those that have met their goals or are no longer appropriate for today's science. Recent plans focused on stroke, epilepsy, Parkinson's disease, and Alzheimer's Disease-Related Dementias. Among recent initiatives:

- the Stroke Trials Network will determine more quickly and at less cost what treatment, prevention, and rehabilitation strategies work best.
- new Epilepsy Centers without Walls will target Sudden Unexplained Death in Epilepsy (SUDEP) and disease modification or prevention.
- the Parkinson's Disease Biomarkers Program is developing assessment tools that will overcome roadblocks to more effective clinical trials.
- the International TBI Research Initiative, coordinated with the European Union and the Canadian Institute of Health Research, will answer questions on care and classification of TBI that have confounded development of interventions.
- two major cooperative studies are investigating the long-term changes in the

brain years after a single TBI or multiple concussions, coordinated via the Foundation for NIH's Sports and Health Research Program, which was established with a donation from the National Football League.

- the NeuroBioBank, NINDS Human Genetics Repository, Federal Interagency TBI Research database, Common Data Elements Program, and an epilepsy clinical genetics data repository are examples of new and continuing resource initiatives that empower individual investigators and promote data sharing.

Finally, and most ambitiously, the President's Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative will dramatically improve tools to understand heretofore unapproachable questions about how networks, or circuits, of brain cells enable us to perceive, think, and act. There are many reasons for confidence that this basic research initiative will ultimately advance progress against disease. Autism, dystonia, and epilepsy, for example, are fundamentally disorders of brain circuitry, and stroke, Parkinson's, and Alzheimer's disease disrupt brain circuits as nerve cells die. Even with our limited understanding of brain circuits and imprecise technologies for altering them, interventions that compensate for malfunctioning brain circuits already produce remarkable results. For example, DBS reverses symptoms for many people with Parkinson's disease and dystonia, and paralyzed people have controlled a robotic arm by signals directly monitored from their brains' movement control circuits. It is perhaps obvious that better understanding of brain circuits and tools to influence their activity would greatly improve these interventions, but history teaches that the most important payoffs of the BRAIN

Initiative, as for basic research generally, may be entirely unforeseen.

Mr. HARRIS. [Presiding] Thank you very much, Dr. Collins. And I am humbled to chair the hearing with such an esteemed panel as I think the ranking member has said. And I could listen forever to that. You know, it has been 10 years since I have been in a laboratory.

Dr. COLLINS. Come on back.

[Laughter.]

Mr. HARRIS. Well, no, I can come in, but I do not recognize anything that is there. You know, as I tell people, when I was delivering obstetric anesthesia, of course, you know, narcotics were part of the delivery. We could never understand, like, why some women needed more, some women needed less. And, you know, I think genetics are going to actually play a role, going to make the field that I was in much safer when we look to how to deliver things worldwide, make it safer worldwide, not just the United States.

So I am going to open the questions, and my first is a great deal of concern because I do think that the NIH is the engine of biomedical basic and early translational research, and that is what it ought to be. And, you know, we have had this conversation. That is what I think the focus ought to be.

THE PRESIDENT'S BUDGET

So I am very concerned about what the President's budget does to it because, you know, the Secretary was here last week saying—or I guess 2 weeks ago—saying that the budget “reflects the Administration's priority, the NIH budget, to invest in innovative biomedical and behavioral research that advances medical science while stimulating economic growth.” And says that you can show that because there was an increase of \$211,000,000.

But when you go to the strategic goals sheet for your budget that says what are the various strategic goals—and it is to strengthen healthcare, advance scientific knowledge and innovation, advance the health and safety and well-being of the American people, and increase in efficiency in HHS—the advanced scientific knowledge and education line actually was cut by a \$1,000,000,000, a 4 percent cut. And the money was redistributed to emphasize primary and preventive care linked with community, which went from \$1,000,000,000 to \$1.6 billion, and ensure program integrity and responsible stewardship of resources, which went from \$1,000,000,000 to \$1.5 billion.

So, in fact, although the Secretary said the Administration's priority is to invest in innovative biomedical research, there was a 4 percent actual dollar cut. We are not counting for inflation. So I am going to ask you, Dr. Collins, what is emphasized primary and preventive care linked with community because I do not even understand what that is, much less why it took \$600,000,000 out of advancing scientific knowledge and innovation. Could you enlighten me on this? I do not even know what that means.

Dr. COLLINS. Well, Dr. Harris, thanks for the question. And I am not quite sure exactly what this means either because this is a very high level representation of budget priorities put together at the Department level. Certainly at NIH's level, we have our own very clear ideas about how to try to make the most of the budget that

has been proposed for Fiscal Year '15, recognizing that because of the Ryan-Murray envelope, it is going to be quite constrained.

Mr. HARRIS. So do you believe that we should be taking \$1,000,000,000 out of advanced scientific knowledge and innovation and putting more than half of that billion into emphasize primary and preventive care linked with community. I have been in the medical field for a few years. I do not understand what that is. I do not understand what the NIH is doing with that. So you may have to get back to me.

Now, the other one is increase efficiency, and this is the NIH. This is from the budget document we got. One category is "increase efficiency, transparency, and accountability of HHS programs." I am assuming they are talking about the NIH. "Ensure program integrity and responsible stewardship of resources," goes from \$1,000,000,000 to about \$1.6 billion. Again, you know, if the Administration said, okay, let's put another \$2,000,000,000 into advanced scientific knowledge and we will spend a little more here. But they did not. They redistributed it.

What in the world is "ensures program integrity and responsible stewardship of resources?" I mean, I just do not understand. Have you seen this document?

Dr. COLLINS. I confess this is not a document that I have paid much attention to, Dr. Harris.

Mr. HARRIS. Thank goodness because if you pay attention to this, you are taking \$1,000,000,000 out of cancer research and the genomics research that you are doing. And, you know, it is of grave concern to me that that decrease is occurring.

Dr. COLLINS. Well, let me say that I think, in general, the Department has been recognizing the fact that the NIH as a scientific agency is in the best position to be able to decide what the priorities should be in the face of a given budget allocation.

Mr. HARRIS. So you believe you will not have to comply with these strategic goals.

Dr. COLLINS. I am not sure exactly how those goals would be defined if one had to put a definition on it. But as—

Mr. HARRIS. That makes two of us because I do not know what this means.

Dr. COLLINS. Yes, this does not seem like it overlaps particularly well.

Mr. HARRIS. Now, even if we took that aside with the tap. You know the tap increase is proposed to be \$150,000,000 more coming from the NIH going into whatever, non-NIH things. Well, I am sorry, I think you have National Library of Medicine there. And I will follow up in another round, but that only leaves a \$50,000,000 increase. And again, it appears all that increase goes to these other things besides—I mean, again, this is striking to me, a \$1,000,000,000 cut. I am just shocked by it. Anyway, but thank you very much.

Ms. DeLauro?

Ms. DELAURO. Thank you. Thank you very much, Mr. Chairman. Since, Mr. Chairman, you are going to talk about evaluation taps in a different round, I would be happy to address that issue in a different round as well. So let me move to a question that I have.

INCLUSION OF WOMEN IN CLINICAL TRIALS

Dr. Collins, 20 years ago, Congress passed the NIH Revitalization Act of 1993, directed NIH to conduct research on diseases and conditions that primarily affect women, and required an appropriate number of women and minorities to be included in all NIH-sponsored clinical trials. We worked very, very hard on that, and I worked with my colleague, Mrs. Lowey, again in a bipartisan way to do that.

NIH guidelines now, as I understand it, with limited exceptions, require women to be included in phase three research trials. Unfortunately, the guidelines do not require female cells or animals to be included in phase one and two of clinical research. In phase-three trials, females are not represented proportionately to the disease prevalence. The first step toward personalized medicine is having a better understanding biological sex and gender differences. Regrettably, we still have a lot of work to do in that regard.

Cardiovascular disease, the leading killer of women in the U.S., affects men and women differently at every level: prevalence, underlying physiology, risk factors, presenting symptoms, outcomes. A 2009 review of cardiovascular disease device clinical trials found that only one-third of trial subjects are female, fewer than 31 percent of the trials that included women reported outcomes by sex. True about depression. American women are twice as likely as men to suffer from depression, yet fewer than 45 percent of animal studies on anxiety and depression use female lab animals.

Questions. Percentage of NIH basic research in phases one and two of clinical research that includes female representation. What do you perceive as barriers to increasing female representation in the research so it is proportional to disease prevalence? What can Congress do to help you facilitate increasing female representation in basic research in phases one and two of clinical research?

I will mention the brain piece as well at the moment because very, very exciting development in research what you are doing here. Will the BRAIN Initiative be mapping a male brain and female brain? If not, why not? How will the BRAIN Initiative data be collected, analyzed, and made available?

I really want to get to the questions that have to do with the research and the involvement of women.

Dr. COLLINS. Great. Thank you for the question, and I will give a quick response and maybe ask Dr. Gibbons to say a quick word about cardiovascular disease and Dr. Landis about the brain.

But first of all, we do keep track of participation in clinical trials, males and females. Currently in Fiscal Year '13, 57 percent of those enrolled in NIH clinical research were women; 73 percent of those involved in phase three clinical trials, phase three trials, were women. So we are responding to the very serious issue that was pointed out 20 years ago by the Congress about the fact that women were, in fact, not involved. And thank you, Mrs. Lowey, and thank you, Senator Mikulski, and others who brought that to our attention, of course, leading to the Women's Health Initiative and many other issues.

But we have not achieved a perfect outcome. The recent information about dose differences for Ambien, for instance, raises that question again. And, of course, we do not control the sex representation in trials that we do not fund.

I would say in terms of animal models, you have a serious point there that oftentimes those who are working with animal models choose to use males, and the reasons for that are not compelling. They are reasons about estracycles that are considered to be variables, but, in fact, we need to do something about that.

Dr. Janine Clayton, who is the head of the Office of Research on Women's Health, and I are actually going to submit very soon a commentary exhorting the animal models folks to change this practice because we are missing out on information we need to have.

Very quickly, Dr. Gibbons, about cardiovascular disease.

Dr. GIBBONS. Well, certainly the NHLBI has had a long tradition of inclusion in its research projects. Indeed, the iconic Framingham study back in 1949 included both men and women, and that has been true of all of our cohort studies that represent diverse parts of our population. And certainly, with the lead of the Women's Health Initiative, which is a game-changing clinical trial involving women, we have other studies looking at coronary disease in women, the Wise study, for example, to show the different sort of pathobiology of that disease in that group.

Similarly, we continue to enhance awareness among women that indeed it is the leading cause of death, five-fold greater than breast cancer. So we are very much engaged in ensuring that we have an impact on women's health.

Dr. LANDIS. So the BRAIN Initiative—

Mr. HARRIS. They may have to wait until the next round. Keep your thoughts. Hold your thoughts, Doctor.

Ms. Roby.

REPRODUCIBILITY OF RESEARCH FINDINGS

Ms. ROBY. Thank you all for being here today. Dr. Collins, I was pleased to see that NIH is emphasizing the importance of experimental rigor and transparency of reporting of research findings in order to increase the ability for other scientists to replicate that research. And we can all surely agree that ensuring public trust and integrity of the scientific enterprises requires rigor, reporting, and accountability.

Last year, an article in the Washington Post made assertions that according to the National Academy of Sciences, that the percentage of scientific articles retracted because of fraud increased ten-fold since 1975. I understand the redactions are higher than the number that are reviewed and referred to the HHS Office of Research Integrity and eventually pursued last year.

NIH has the authority to require repayment for the cost for material violations of the cost principles and other terms and conditions of NIH-funded awards. How many times in the past 5 years has NIH actually used that authority for any research? And if so, you know, based on that number of times, how much was required to be repaid and then actually repaid to the Federal government?

Dr. COLLINS. So, Ms. Roby, it is a very important, and we are obviously deeply concerned if we encounter situations where fraud

has been perpetrated in the publication of science because science is all about determining the truth and making sure that one is completely objective about reporting that.

I will have to answer for the record your question about how many times NIH has had the opportunity to recover funds as a result of a claim or a conviction of the conduct of fraud by an investigator. Obviously the Office of Research Integrity, which is part of the Department of Health and Human Services, oversees those investigations, and they report publicly on when they have identified an individual who is often then disbarred from further research applications for a period of time. But I do not know the total dollar——

Ms. ROBY. If you would get that to me, I would appreciate having that information. Does it apply to intramural researchers as well?

Dr. COLLINS. I do not know of an example where there has been a cost recovery from intramural, but I will find out for you.

NIH- Roby – Research Integrity

Thank you all for being in here today. Dr. Collins, I was pleased to see that NIH is publicly emphasizing the importance of experimental rigor and transparency of reporting of research findings in order to increase the ability for other scientists to replicate that research. And we can all surely agree that ensuring public trust and integrity of the scientific enterprises requires rigor, reporting and accountability.

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If you could get that to me, I would appreciate having that information. Does it apply to intramural researchers as well?

Answer:

In responding to the specific issue of research integrity, as well as the larger issue of compliance, it is important to recognize the responsibilities of NIH grantees when accepting award funds. Under statutory direction from Congress, organizations that apply for NIH grants bear the responsibility for ensuring the integrity of the research conducted in their name. Applicant organizations must provide an assurance that they will comply with the terms and conditions of award and policies found in the NIH Grants Policy Statement, including having adequate policies and procedures in place to investigate instances of scientific misconduct. Principal investigators are designated by the applicant organization to have the appropriate level of authority and responsibility to direct the project or program to be supported by the award. Thus, in assessing a grantee's compliance under circumstances in which an investigator has been found to have committed scientific misconduct, the actions of the organization leading up to and during the investigation are important considerations.

When NIH undertakes enforcement actions it is done in accordance with applicable statutes, regulations, and policies. NIH generally will afford the grantee an opportunity to correct the deficiencies before taking action, unless public health or welfare concerns require immediate action. However, even if a grantee is taking corrective action, NIH may take proactive actions to protect the Federal government's interests, such as precluding the grantee from obtaining future awards for a specified period or monitoring grant activities more closely to prevent future non-

compliance. NIH generally will suspend (rather than immediately terminate) a grant; however, NIH may decide to terminate a grant if the grantee does not take appropriate corrective action during the period of suspension. NIH may immediately terminate a grant when necessary, such as to protect the public health and welfare from the effects of a serious deficiency.

If a grantee fails to materially comply with the terms and conditions of award, NIH may take enforcement action(s), including disallowing costs, withholding further awards, or wholly or partly suspending the grant, pending corrective action. NIH may also terminate the grant for cause. Special award conditions may be included to require correction of identified financial or administrative deficiencies, as a means of protecting NIH's interests and effecting positive change in a grantee's performance or compliance.

NIH may withdraw approval of the Project Director/Principal Investigator (PD/PI) or other senior/key personnel specifically referenced in the notice of award if there is a reasonable basis to conclude that the PD/PI and other such named senior/key personnel are no longer qualified or competent to perform the research objectives. In that case, the awarding NIH Institute or Center may request that the grantee designate a new PD/PI or other named senior/key personnel. Other actions available to NIH include suspension or debarment of either an organization or individual under Government-wide Debarment and Suspension rules.

Non-compliance may include rare instances of confirmed research misconduct, as well as instances of failure to comply with any number of policies found in the NIH Grants Policy Statement. The nature of the enforcement action depends on the severity and duration of the non-compliance. The NIH's grants management offices, located in each of the NIH Institutes and Centers, are responsible for managing and overseeing NIH grant awards. They also have the authority to impose enforcement actions.

In addition to enforcement actions, NIH can and does make changes to the terms and conditions of awards for scientific reasons. For example, if recruitment in clinical research has been delayed, or if disruptions have occurred due to a natural disaster, NIH's grants management offices may make changes to the original terms and conditions. Thus, temporary suspensions or changes in funding can occur for scientific reasons, rather than as a result of non-compliance. Distinguishing changes in award conditions that result from changes in scientific conditions versus those enacted to correct non-compliance would require careful manual review of each grant award over the last five years, which would involve more than 150,000 grant awards. If this were a feasible task, which it is not, the resulting data would likely be error prone given the high degree of skilled interpretation needed to distinguish changes made for scientific reasons versus those made due to noncompliance.

With regard to the NIH intramural research program, an investigator found to have committed research misconduct would be subject to the same disciplinary actions otherwise available under applicable personnel policies (e.g., supervision, suspension, termination). The investigator

would also be subject to HHS Administrative Actions brought by the HHS Office of Research Integrity (ORI).

Ms. ROBY. That would be great, too. And in your January 2014 Nature article, you stressed that poor training is the problem for the increased lack of scientific integrity. And so, how did you ascertain that conclusion, and do you have data to support that?

Dr. COLLINS. So let me make a really clear distinction here between what we would consider as committing scientific fraud, which is an intentional misrepresentation of the truth, versus an issue where a published paper cannot be reproduced by another group, and there are all kinds of reasons why that might be—different conditions, different strain of animal, different buffers, all of the things that you can imagine would make it hard to reproduce, and yet, the intentions of the investigation were completely honorable. I would not call that a breach of scientific integrity. That is a problem with somehow the way in which studies are designed or reported. Are the details all there?

The article that Dr. Tabak and I wrote in January was really about this issue of reproducibility because it is terribly wasteful to have a study published, and then it turns out that it cannot be reproduced. And in that instance, one of the problems that we discovered is very much a reality, is that many of the scientists who are doing particularly animal studies where they are testing a new possible therapeutic have not had the training in terms of how to design that kind of study. How many animals should be used, male and female? Should they be blinded to the investigator in terms of which animal got the treatment and which did not? Are the statistics being done in the most rigorous way?

It is clear that some of those studies have not lived up to those kinds of principles, and that is the training that we are aiming to try to introduce into the system.

RIGOR AND TRANSPARENCY OF REPORTING

Ms. ROBY. Okay. Real quickly—my time is running short—can you highlight for me in your 2015 budget request where you will stress the importance of experiment rigor and transparency of reporting these research findings, and how will that success be measured?

Dr. COLLINS. So we will be introducing a training program for graduate students and post-doctoral fellows to improve the rigor in these kinds of analyses. We will be tracking then to see what, in fact, are the consequences in terms of the ability to reproduce experiments. Many of the institutes have initiated pilot projects on their own in this area of reproducibility. We have quite a long list of those. We will be evaluating those to see which ones worked, and then trying to expand the ones that seem most successful.

Ms. ROBY. Thank you. My time has expired.

Mr. HARRIS. Ms. Lowey, the ranking member?

Ms. LOWEY. Thank you, and welcome again. And before I get to a question, I just want to follow up on my colleague, Rosa DeLauro's, comment because when I got on this committee, as was said, 20 years ago, I was alarmed to find out that only male lab rats were used in research. So I kind of assumed this was straightened out, and I do hope you get back to us in response to the letter we sent because this is so important in terms of the research you

do and the real life clinical trials that are taking place. So I thank you.

Dr. COLLINS. Yes, we will get that letter to you.

[The information follows:]



DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

APR 01 2014

National Institutes of Health
Bethesda, Maryland 20892

The Honorable Nita M. Lowey
U.S. House of Representatives
Washington, DC 20515

Dear Representative Lowey:

Thank you for your letter expressing your concerns that basic science research is predominantly conducted in male animal models of disease. I appreciate your long-standing support of the NIH and your commitment to improving women's health. As I mentioned in our discussion at the hearing last week, I realize the NIH needs to do a better job of including female animal models in pre-clinical research. We are committed to addressing this issue.

In your letter of January 17, you asked that we conduct a thorough review of the representation of male and female animals in NIH-funded basic research. Detailed information on the proposed use of animals, including the number as well as their sex, is requested in grant applications. However, since this information is submitted in a narrative form, we would be required to review and extract information from thousands of individual grants. In order to provide timely and meaningful data to respond to your query and inform our policies and programs, we will draw a sample of grants that propose the use of vertebrate animals and assess the inclusion of male and female animals. I will send you those data as soon as they are collected.

I believe that NIH research must work to uncover the "whys" and "hows" behind sex and gender differences in health. This is a personal priority for me. We are working internally to determine how best to enhance the investigation of sex differences in funded research, both in clinical research and in preclinical research involving animal models. This is a complex issue and the NIH is approaching it in a thoughtful and rigorous manner.

In coming weeks, Dr. Janine Clayton, the Director of the Office of the Research on Women's Health, and I will publish a commentary highlighting the importance of including female animal models in preclinical research and urging researchers to avoid exclusive reliance on male animal models.

Again, I thank you for your support of the NIH and for your continued interest and dedication to women's health. I hope the information about our new efforts is helpful, and I will update you on our progress.

An identical letter is being sent to The Honorable Rosa DeLauro.

Sincerely,

Francis S. Collins, M.D., Ph.D.
Director

VACCINATION RATES

Ms. LOWEY. I want to ask you to comment—I think it is Dr. Fauci—that in recent days there has been at least 20 confirmed cases of measles in New York, including nine in children. Only three of the 11 infected adults had records proving they were vaccinated. This outbreak does highlight to me what happens when individuals choose to not use vaccine. And I think it is very important to see if they are choosing it out of ignorance, poverty, not having the information, not be encouraged, or not choosing it because of some—which have been proven false by you—claims, such as thimerosal, et cetera, causing autism.

Can you tell us what are you doing to increase vaccination? If you can clarify that.

Dr. FAUCI. Well, first of all, let me just clarify the incident and the experience that you are referring to in New York. You are absolutely right on the numbers that you gave. It has been our experience when we do surveys that the individuals who do not get vaccinated are not vaccinated because of an unfounded fear of adverse events associated with vaccines that are either completely non-existent or are so rare that they barely register when considering the risk-benefit ratio.

People forget measles, and that is one of the issues where you are a victim of your own success. I got measles when I was a child, and my sister got measles. At that time, before the availability of the measles vaccine in 1963, we had about four to five million cases a year in the United States with 500 deaths, 48,000 hospitalizations, and a thousand children per year with chronic disabilities due to measles.

When the measles vaccine came out, with great enthusiasm people wanted their children vaccinated because they had a memory of the devastating effect of the disease. What happens when you have a triumphant public health success as we have had with measles elimination is that people forget because they do not have the corporate memory. And then when you have that insidious disinformation about the adverse events of measles vaccine being worse than the disease, then you unfortunately have mothers who read or hear that disinformation and do not have their children vaccinated.

So we must remember that one of the most successful extrapolations of basic research to a defined intervention is vaccination. And a good example, Ms. Lowey, is that in the United States we are down to 90 percent of people vaccinated. That is not good enough. In the UK, they are down to 80 percent. And the United Kingdom has frequent outbreaks of measles, more than just 20 children at a time, often hundreds of individuals at a time.

The measles vaccine is 99 percent effective when you get it with the first dose at 12 months and the second dose at 4 to 6 years. It seems such a shame and a tragedy for those children who get infected with a disease that could have dire consequences because of disinformation and misinformation about vaccines.

Ms. LOWEY. So we have to do more to get the word out. Okay. Now, let me just say I am pleased that the budget deal allowed us in Fiscal Year 2014 to restore some of the damage done to the NIH

by sequestration. But as you know, we were not able to fully replace the cuts, and instead fell short by a little more than \$700,000,000. Your budget for 2015 includes a small increase of \$200,000,000. This is far below the level that we need to be investing in biomedical research.

ADDITIONAL ACTIVITIES W/ADDITIONAL RESOURCES

So in the little time that is left, who would like to tell me what sort of research activities that the NIH could be pursuing if additional resources were available?

Dr. COLLINS. Well, goodness. In the little time available, there must be hundreds of things from cancer to diabetes to heart disease, infectious diseases, the BRAIN Initiative, autism, Alzheimer's. All of these are areas where we have great research potential, but we are not going as fast as we could.

Ms. LOWEY. Okay. That red light is a red light to make it clear we have to invest more in the NIH, right, Doctor?

[Laughter.]

[The information follows:]

NIH- Lowey - Investing in Biomedical Research

So we have to do more to get the word out. Okay. Now let me just say I am pleased that the budget deal allowed us in FY 2014 to restore some of the damage done to the NIH by sequestration. But as you know, we were not able to fully replace the cuts, and instead fell short by a little more than \$700 million. Your budget for 2015 includes a small increase of \$200 million. This is far below the level that we need to be investing in biomedical research. So in the little time that is left, who would like to tell me what sort of research activities that the NIH could be pursuing if additional resources were available? And submit the rest for the record.

Answer:

The FY 2014 increase enabled NIH to increase support for biomedical research. These funds will enable the NIH to support research to understand the basic biological causes of disease and to develop treatments for a great many of the diseases and disorders that exact a tremendous toll on society, including Alzheimer's disease, autism, heart disease, cancer, and infectious diseases. NIH-supported researchers make advances in these areas every single day, and additional resources facilitate investment in many of the hundreds of diseases in the NIH portfolio as well as increase the pace of scientific discovery. A steady source of funding helps support biomedical research. Science is not a sprint but rather a marathon that requires sustained support over many years. Adjusting for inflation using the biomedical research price index (BRDPI) NIH purchasing power has declined 20 percent since 2003. Increased investment in NIH would help to reassure scientists that they will have the necessary support for the duration of their projects. Additional resources would lead to more grants awarded to fund investigator-initiated research, thereby allowing the country's most innovative scientific thinkers to chart the best path forward in their research areas. Increases could be used to support research examining basic mechanisms of biological and behavioral function, such as the projects in the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative. BRAIN supports fundamental research aimed at understanding how individual brain cells and complex neural circuits interact, with the ultimate goal of finding new ways to treat, cure, and even prevent neurological and psychiatric conditions. Increases would raise the number and variety of projects that NIH may support in this and other related areas of research.

Capitalizing on knowledge gleaned from additional investments in basic research could also hasten the translation of that research to improve human health. Opportunities abound to potentially shorten the time to develop a universal flu vaccine or to expand research efforts in immunotherapy to treat cancer patients. Funds could also be leveraged in partnerships with the private sector, similar to the new Accelerating Medicines Partnership (AMP) that brings biopharmaceutical companies and several non-profit organizations together with the NIH to identify and validate biological targets of disease. The ultimate goal of the AMP is to increase the number of new diagnostics and treatments and to decrease the time and cost of their development.

Furthermore, resources would enable the NIH to strengthen the scientific research workforce and to build the scientific infrastructure of tomorrow. Increasing the number of fellowships and trainees funded by NIH would ensure that the most promising young scientists have the necessary support at the outset of their careers to prepare them for their future in biomedical research.

Our country has never faced a time of greater scientific opportunity than the present. Timely and stable funding will help us to capitalize on this promise to advance our health, knowledge, and economy, as well as maintain our global leadership in research and development.

Mr. HARRIS. And submit the rest for the record. Mr. Joyce.

COPE: THIRD LEADING CAUSE OF DEATH

Mr. JOYCE. Thank you, Chairman, Dr. Collins. And perhaps this is best to address to Dr. Gibbons. But with COPD being the third leading cause of death in the United States, I understand the National Heart, Lung, and Blood Institute is the leading body to address this disease. We must develop more precise, personalized, and effective therapies to preempt chronic disease. Please describe the work that you are doing on COPD and the impact of that work on public health.

Dr. GIBBONS. Thank you, Congressman. One more time, okay. Thank you for that question. As you pointed out, COPD is a major burden on the American people, a leading cause of death and disability. And it is an area in which there is still much to be learned by further research.

It is also an area in which there is great promise for more personalized precision medicine. When I was in my training, we just had a very crude notion of this chronic lung disease. But now with the advances in genetics, we are starting to have an appreciation for the various subtypes of this disorder in ways of identifying the actual biological pathways that promote it.

For example, the recent discovery of this MUC5B gene that appears to play an important mediator role and an appreciation now for the role of the immune system in its pathogenesis. And that is providing an opportunity for us to intervene and identify patients at greatest risk and get the right drug to them at the right time in ways that can change that natural history.

INVESTMENT IN AGGRESSIVE CANCERS RESEARCH

Mr. JOYCE. Thank you. Dr. Collins, if you will, a number of Federal bills recently introduced target specific funding for diseases such as breast cancer and pancreatic cancer. What is the optimal appropriation level, and who should we best look to to decide the level of funding to better invest in aggressive cancers that we have not made significant progress in reducing the incidents of or mortality of the disease, such as pancreatic cancer, glioma, or small cell lung cancer?

Dr. COLLINS. I appreciate the question. I am going to ask my colleague, Dr. Varmus, to say something about this since he oversees the cancer research effort. I guess the overarching principle, there are so many needs for research across many diseases, including all the subtypes of cancer, that it does make us somewhat uneasy if there is a top-down effort to try to identify one as being more important than the other as opposed to looking at the scientific opportunities and the public health need. But I will ask Dr. Varmus to speak.

Dr. VARMUS. Thank you for the question. The legislation we are responding to does not give us a specific number for the amount we should be spending on these diseases. Instead, what it proposes is something that is very dear to the heart of most NIH Institutes, and that is to take a problem against which we are not making rapid progress, bring people together from various fields that im-

pinge on that problem, and try to come up with some new suggestions for things to do.

In the case of the Recalcitrant Cancer Act that was passed a year and a half ago, we were asked to do this specifically for certain diseases that meet certain public health criteria. And we have already submitted to Congress a report on pancreatic ductal adenocarcinoma, in which we outlined four important things, some of which we had already started, some of which we had in our distant sights, a couple of which were new as a result of the workshop.

For example, we have built a new program to study one gene, which is implicated in over 95 percent of pancreatic adenocarcinomas, a project we are carrying out at the Frederick National Lab in Frederick. Secondly, we are emphasizing more a topic that Dr. Collins introduced, the use of immunological therapies in pancreatic cancer. Third, we are making note of an important new observation about the frequency with which pancreatic cancer is diagnosed after a diagnosis of diabetes mellitus, type 1. And we are also taking advantage of some new risk factors we had not previously appreciated that come from both genetics and from pathology with the appearance of cysts in the pancreas.

So those are projects, some new, some sort of new, that we are pursuing more aggressively without worrying about the actual dollars, but worrying about these tasks and trying to find people to pursue them.

Mr. JOYCE. I for one certainly hope you succeed, Doctor. I yield back.

Mr. HARRIS. Thank you very much. Ms. Lee.

Ms. LEE. Thank you very much. And let me once again reiterate everyone's real appreciation for the work that all of you do each and every day. It really is about not only saving lives, but enhancing the quality of life for each and every one of us.

I just wanted to mention a couple of things. I have a mother who is 89 with COPD. And I have recently learned that COPD is the third leading cause of death in the Nation, and that is one issue I would like to ask Dr. Gibbons about in terms of the latest research as it relates to chronic, obstructive pulmonary disease. That is one issue.

MINORITY RESEARCHERS

Second is in terms of the minority researchers at NIH, in 2011 NIH—and thank you very much for that, Dr. Collins—you commissioned a study which showed that minority researchers receive fewer of the R01 grants than their counterparts, which is an issue that we continue to raise with NIH. So I was pleased to see that you announced the funding for the new Enhancing the Diversity of the NIH Funded Workforce Program. And I would like to just get a status of these new programs, what the funding level is in the budget that you have requested, and what the subcommittee can do to support that initiative.

Also I wanted to ask, yes, I guess, Dr. Collins, you being the person to know about this issue around the National Institute of Minority Health and Health Disparities as it relates to the, well, the fact that it did not receive an increase where the other institutes, I believe, received somewhat of an increase. The fact that minority

health disparities continue to be a big issue, you know, to me warrants a larger increase in its budget to ensure that the work is completed or at least is on par with the other institutes.

So I am wondering what happened and what the decision was not to ask for an increase in funding for the Minority Health and Health Disparities Institute.

CHRONIC OBSTRUCTIVE PULMONARY DISEASE

Dr. GIBBONS. With regard to chronic obstructive pulmonary disease, as you mentioned, a very common disorder, it is clear that the NIH over the years has done a number of intervention trials to change the natural history, documenting the importance of providing oxygen therapy, for example, and improving care options that include bronchodilators, antibiotics, and so forth.

More exciting is the work in the COPDgene study that is really helping us to dissect out the different subsets of patients most likely to respond to those therapies. And indeed, a recent study is using imaging techniques, finding out who is at greatest risk for the progression of the disease. And it is the identification of these particular high risk groups that will be critical for us changing that natural history.

Ms. LEE. The cessation of smoking, of course, is the number one preventive behavior change.

Dr. GIBBONS. Absolutely.

Ms. LEE. Also in terms of just the third leading cause of death, that is a fact. I mean, when I conducted the research for this hearing, I did not know that. I mean, I know the disease from a practical standpoint, but I had no idea.

Dr. GIBBONS. It is a very common disorder predisposed by exposure to tobacco smoke, as you mentioned. But even after the individual stops smoking, there is often a continual inexorable decline of lung function. We are still trying to understand why that is. And so, although we want smoking cessation, it will be critical to understand how the lung can better repair itself.

And so, indeed there is basic science discovery work being done to actually put lung cells on a chip so we can understand more about the biology of the lung and how it responds to a toxin like tobacco.

Ms. LEE. Thank you. And still no cure. Dr. Collins? Thank you very much, Dr. Gibbons.

RECRUITMENT AND RETENTION OF MINORITIES

Dr. COLLINS. So this 5-minute rule is brutal, is it not, so let me quickly try to respond to your other two very important questions. Yes, based upon the extensive work done by a working group of my advisory committee to the director, we have a variety of new programs to try to do something to increase the recruitment and retention of minorities into our scientific workforce.

The flagship of those programs right now is one called BUILD, which aims to try to provide for underrepresented individuals who are currently not coming into our workforce in substantial numbers, real scientific experiences during the summertime during their college years, and also for a year or two after college to see if this is a good fit for them before going onto graduate school and

a career in science. This program will be combined with another program on mentoring because clearly mentoring is in short supply for many people who are not from the majority groups. And also a careful evaluation program to see what is working and what is not.

We are waiting for the applications to the BUILD program to be received. They are due coming up in April. We are very excited about this program, and we will be spending about \$50,000,000 a year on this combination of programs, which are quite different than anything we have tried before. And we are excited about seeing where they lead.

Ms. LEE. Thank you, Mr. Chairman. Will we have a second ground?

Mr. HARRIS. Yes.

Ms. LEE. Okay. Thank you very much.

Mr. HARRIS. Ms. Roybal-Allard.

Ms. LEE. Thank you.

Ms. ROYBAL-ALLARD. Dr. Collins, last August, I along with 80 of my colleagues in the U.S. House of Representatives sent you a letter expressing our support for NIH funding of social, behavioral, and economic research, which is clearly a link to the Agency's mission and is essential to understanding the role that socioeconomic status plays in the onset, progression, treatment, and prevention of disease and disability. And to date we have not received a reply to our August letter.

OPPNET

Also, we continue to hear reports from advocates that health economist staff positions at NIH are not being filled when people resign, and that fewer institutes are participating in OppNet, the only trans-NIH initiative focused on basic behavioral and social science research.

My questions are, is the interdisciplinary research, including all the social, behavioral, and economic sciences, still an important part of the overall NIH mission? What is the participation level in OppNet at this point? And is the NIH consciously attempting to downsize the number of staff economists by not replacing positions that have been vacated? And if so, why given that NIH support of behavioral and social science research has yielded important scientific advances and, in some instances, significant cost savings?

Dr. COLLINS. Well, thank you for the question. My apologies that you have not received a response to the letter that you mentioned. That is, quite frankly, an error on our part, and we will be getting you that response. I read the letter when it came in. I thought it was very thoughtful, but somehow we never quite got into the system a response. And that does not usually happen, and it is my mistake, and sorry about that.

Ms. ROYBAL-ALLARD. But we will be getting a letter?

Dr. COLLINS. You will be getting a letter, yes. And again, it should have come much sooner than this.

[The information follows:]



DEPARTMENT OF HEALTH & HUMAN SERVICES

Public Health Service

National Institutes of Health
Bethesda, Maryland 20892

APR 03 2014

The Honorable Lucille Roybal-Allard
U.S. House of Representatives
Washington, DC 20515

Dear Representative Roybal-Allard:

Thank you for your August 6, 2013, letter expressing support for behavioral and social sciences research at the National Institutes of Health (NIH). Please accept my apologies for the long delay in responding to your letter.

All of the NIH Institutes and Centers support activities in behavioral and social sciences research, totaling over \$3.5 billion in fiscal year 2013. The Office of Behavioral and Social Sciences Research plays an important role in stimulating, initiating, and promoting research to evaluate the role of behavioral, social, and lifestyle factors in the causation, treatment, and prevention of illnesses and related public health problems, and advising on these topics with NIH scientists and others within and outside the Federal government.

The NIH recognizes the role of behavioral and social factors in the etiology, treatment, and prevention of a wide range of health conditions. Unhealthy behaviors like smoking, excessive alcohol consumption, and illicit drug use—as well as inactivity and poor diet—raise the risk for many debilitating and costly health problems. Collectively, these behaviors contribute to approximately 40 percent of all deaths in the United States. The development of effective interventions to improve health depends on a robust scientific foundation of research that identifies the psychological, social, and cultural mechanisms underlying behaviors and social circumstances that adversely affect health and how they can be modified to improve health. Behavioral and social sciences research has never been more timely and important, and the NIH sees great opportunities to apply what we have learned toward helping change people's behaviors to improve their health and well-being.

Thank you for your continued support of the NIH, including its important behavioral and social sciences research portfolio.

Sincerely yours,

A handwritten signature in dark ink, appearing to read "Francis S. Collins".

Francis S. Collins, M.D., Ph.D.
Director

Dr. COLLINS. I would say in terms of NIH's commitment to behavioral and social science research, including health economics, that remains strong. We spend \$3.5 billion of our budget each year in behavioral and social sciences research. We have just heard about COPD as an example of how critical it is to try to understand behavior because it does contribute to so many diseases, and the ability to come up with better prevention strategies often depends upon that. And they are an exciting set of new ways to approach this using, for instance, mHealth, the use of cell phones to be able to interact with individuals in real time in a way that encourages healthy behaviors is something about which we are quite excited.

Many other things fit into that space as well, and OppNet is engaged in that space. After all, it is something like 40 percent, and maybe as much as 60 percent, of the risks associated with preventable premature deaths in the United States are because of behavioral choices. And if we are going to be responsible stewards of our mission here to try to prevent unnecessary death, behavioral science has to be part of that.

In terms of your question about health economics, I am not aware of any plan to try to shrink the staffing of that enterprise. I would tell you everything is under a terrible squeeze right now, as you know. We have lost almost 25 percent of our purchasing power for research. That means we have had to cut back in virtually every area. And I am sure all those who are affected by that are sure that their area is getting hit harder than the rest, but nobody is really left untouched by what has been a very difficult 10-year period.

Ms. ROYBAL-ALLARD. Okay. So your commitment remains the same then.

Dr. COLLINS. We are strongly committed to behavioral and social science research.

PANCREATIC CANCER

Ms. ROYBAL-ALLARD. Okay. Dr. Varmus, I want to commend you for the timely appropriation of the scientific framework for pancreatic ductal adenocarcinoma. As you know, I have had a long-standing interest in this issue because of the disturbing fact that pancreatic cancer has a 5-year survival rate of 6 percent compared to the 5-year overall cancer survival rate of 68 percent, and because treatment options and detection methods for pancreatic cancer are extremely limited, as you know.

You mentioned your report, and that there were at least, that I am aware of, four recommendations. One of the recommendations is to research the relationship between pancreatic cancer and diabetes, which I found very interesting. Will the NCI be issuing a funding opportunity announcement in this area? And if so, when will that announcement be made, and how much will be the funding be for?

Dr. VARMUS. Well, thank you for the question. Yes, one of the more interesting discoveries at the workshop was this inter-disease relationship which we think—my light is on. Okay. Thank you. We have a technical problem here. We need another agency to come in and work this out. But one of the more interesting observations

that was discussed at the workshop was this apparent relationship between type 1 diabetes diagnosis and then a subsequent diagnosis of pancreatic cancer.

One of the things this does, of course, is to narrow the cohorts who could be watched to help us in making better diagnostic approaches to early pancreatic cancer. When you have a small cohort of people recently diagnosed with type 1 diabetes, there is a better chance of being able to test the many proposals that have been made over recent years thanks to other programs to improve early diagnosis of all types of cancer in this particular situation, which, as you point out, has been a very difficult one because of the position of the pancreas in the body and our difficulty in seeing pancreatic lesions early.

We have been in discussion with our colleagues——

Mr. HARRIS. Doctor, you are probably going to have to do the rest of that answer for the record. I gave you some extra time, but we really have to keep going.

[The information follows:]

NIH- Roybal Allard - Pancreatic Cancer and Diabetes

Okay. Dr. Varmus, I want to commend you for the timely appropriation of the scientific framework for pancreatic ductal adenocarcinoma. As you know, I have a longstanding interest in this issue because of the disturbing fact that pancreatic cancer has a five-year survival rate of 6 percent compared to the five-year overall cancer survival rate of 68 percent, and because treatment options and detection methods for pancreatic cancer are extremely limited, as you know. You mentioned your report, and that there were at least, that I'm aware of, four recommendations. One of the recommendations is to research the relationship between pancreatic cancer and diabetes, which I found very interesting. Will the NCI be issuing a funding opportunity announcement in this area? And if so, when will that announcement be made, and how much will the funding be for? Doctor, you are probably going to have to do the rest of that answer for the record.

Answer:

We have begun to work on a Program Announcement (PA) that will stimulate research to explore the relationship between pancreatic cancer and diabetes. We expect to issue that PA before the end of the year. The number of awards made in response to this announcement will be contingent upon NIH appropriations and the submission of a sufficient number of meritorious applications. The funds requested and awarded for individual grants are expected to differ, reflecting the needs of the proposed projects.

Dr. VARMUS. Okay.

Mr. HARRIS. Thank you very much. Mr. Fleischmann.

Dr. VARMUS. In preparation, we can discuss—we discuss it later. Thank you for the question.

Mr. FLEISCHMANN. Thank you, Mr. Chairman, and to the entire group here today. I apologize. We have three subcommittee hearings going on concurrently, and I know many of my colleagues experience that same dynamic.

HHS STRATEGIC GOALS

Dr. Collins, I know there has been some discussion about the breakout of NIH funds relative to HHS strategic goals, so I apologize if any of this has already been covered today. But I am very concerned, sir, about the apparent \$1,000,000,000 shift away from scientific discovery and towards “strengthening healthcare.”

It appears that strengthening healthcare funds are not spent on biomedical research or discovery activities as are listed in different categories. I believe this category in the HHS strategic plan is where HHS focuses its Obamacare and healthcare reform activity. Can you assure us, sir, that NIH is not funding any activity relative to healthcare reform or Obamacare?

Dr. COLLINS. Well, thank you for the question. This did come up earlier in a question from Dr. Harris. And I confessed my puzzlement somewhat with the page that is being referred to in terms of how the NIH budget is broken down in these various categories. Happy to assure you that at NIH we are taking our budgetary priorities with great seriousness, and the way in which we intend to spend dollars allocated to us by the Congress reflects scientific opportunity.

We would only, I guess, be said to contribute to something like healthcare reform in the sense that we generate data. That is what we do. We provide evidence. We do research studies. We do clinical trials. We publish the results. And then you can find out from those results what works and what does not. But other than that connection, sir, we are not part of that enterprise.

Mr. FLEISCHMANN. Okay. So the answer then is that you can assure us that NIH is not funding Obamacare other than by collecting data.

Dr. COLLINS. Other than providing a foundation of evidence for good decisions in medicine, we are not.

Mr. FLEISCHMANN. Okay. Thank you. With regard to this funding and whatever activities will be funded in this category, how do NIH and HHS coordinate multi-agency efforts to avoid duplicating work that other agencies are already funded to perform, sir?

Dr. COLLINS. Well, I appreciate that question. And I spend a lot of time as the NIH director trying to be sure that we are making the most of those synergies, avoiding duplication, but also finding areas of significant possible collaboration where we can go faster.

As an example of that, Peggy Hamburg, who is the Commissioner of the FDA, and I set up a Joint Leadership Council where NIH and FDA meet regularly to look at areas that we can work on together. We had a recent meeting of that sort to talk about adding microbial resistance and what to do about a growing problem that both of our agencies have a significant role with. Likewise

with the CDC, we have multiple opportunities and multiple interactions, and Dr. Fauci could tell you quite a bit about those as they relate to public health and infectious disease.

And outside of HHS, we have multiple other connections with agencies, for instance, with DARPA, the Department of Defense's Advanced Research Project Agency, where we have a joint effort right now to develop a tissue chip that can be used for drug toxicity testing. Before you ever give a new therapy to a patient, you can test it on this chip instead. And I could go on and on. A very important part of my job is to be sure that we are looking for those collaborative opportunities and making the most of them, and also with the private sector.

Mr. FLEISCHMANN. Thank you, Doctor. Chairman, I yield back.

Mr. HARRIS. Would you yield me the last of your minute and a half?

Mr. FLEISCHMANN. I will yield, Mr. Chairman.

Mr. HARRIS. Thank you very much. I appreciate that. Let me just follow up a little bit with what was—I think the ranking member who has left, ranking member of the full committee had asked, which is what you could do with that, you know, if your NIH funding was higher. And I guess we cut you kind off halfway through the litany of things.

I assume that the answer would be exactly the same for what you could do with the \$900,000,000, the tap that is being requested in the President's budget. I am assuming it is an identical list, right? It is just let's keep on going with the priorities that we have. Would that be correct?

Dr. COLLINS. Sure. Resources could be utilized in a variety of ways. Another way that this was formulated in the President's budget is this Opportunity, Growth and Security Initiative, \$970,000,000, a similar kind of number. We could expand the BRAIN Initiative. We could go faster on that. We could do more for Alzheimer's disease research to go faster on that.

Mr. HARRIS. So whether we would do it by expanding the total amount or merely decreasing the amount that is taken away for other things, it has the same functional difference in terms of the number of research projects you are going to—

Dr. COLLINS. We would look at our spendable dollars and make the most of what was there.

Mr. HARRIS. That is what I thought. Thank you, Doctor. Mr. Honda.

Mr. HONDA. Thank you, Mr. Chairman, and I want to thank the panelists here. I will be brief in my introduction, but I represent Silicon Valley, and probably one of the things that we recall were the powerful drive of the innovation economy. And, you know, NIH currently supports about 45 projects as a total funding level over \$18,000,000. But there is about \$30,000,000,000 in NIH funding that goes to medical research at all the facilities, and the payback has doubled in our economy across the country. So I guess the logical extension of that is you put more into it, you get more returns on it.

And we talk about NIH turning discovery into health. I am not sure where you draw the line when you want to say, you know, are we going to apply the things that we learned into our healthcare

domain or are we not? That is not a question. It just goes right on, and we apply the things that we learn in our healthcare.

So I think that in terms of money, I think that we should really go back to looking at complete sensible budget rather than looking at how we do things with lists. And the other one is, make do with what you get, and that the position we are in right now, and I really appreciate that effort. And I think that the message really is that we should start looking at our budget in a holistic way so that we have a society that is going to benefit from research, development, and its application to our quality of life.

HEPATITIS B AND C

So in that, I have learned a lot of hepatitis B, and I know that hepatitis B, the greatest sufferers are Asians. The greatest cause of death among Asian Americans in terms of liver cancer are Asian American men. And African Americans are more than twice as likely to be infected with hepatitis C than the Caucasian population. And viral hepatitis is the leading cause of liver cancer, and one of the most lethal and expensive and fastest-growing cancers in this country.

So two questions. One is, what is the National Cancer Institute doing to ensure that liver cancer is a priority, and that is a money question probably. And as a result, you know, with other commitments to research and development we have seen hepatitis C antiviral drugs come to the market in recent years with more expected in the pipeline. So, NIH, what are you doing to ensure that research for hep B drugs remain a priority so that we can find a treatment and ultimately a cure, because we have achieved that for hep C? Hep B we are real close. So I would like to know how you are going to arrange your limited resources to that end.

Dr. COLLINS. Well, we have the right people to answer those questions. Dr. Varmus can say something about liver cancer, and Dr. Fauci can tell you some exciting news about hep C.

Dr. VARMUS. Mr. Honda, that is a very timely question because the NCI just last week conducted a day and a half workshop on liver cancer, which remains worldwide a very important cause of mortality from cancer. And as you probably know, we have been successful over the years in defining major risk factors for liver cancer—hepatitis C and hepatitis B infections, exposure to certain toxins in food, like aflatoxin, and recently obesity and diabetes have proven to be important risk factors as well.

The hepatomas, the liver cancers that arise, arise by somewhat different pathways. We are making a clear statement that we are interested in pursuing more vigorously some of those opportunities. I will let Dr. Fauci talk in a moment about the exciting new work done to treat hepatitis C virus. There is the prospect in the long run of a hepatitis C vaccine.

One of the things that emerged in our meeting was that although hepatitis B vaccine has been available for a long time and actually is quite cheap, it is not being used as effectively and as universally as many of us had thought. And some studies of why that vaccine is not better used need to be undertaken. So we are totally on board with your sense that this is a very important cancer against which we have important information, and a number of things will

be done over the next couple of years to try to ensure that people do not think that liver cancer is a solved problem because we have a vaccine against hepatitis B and now drugs against hepatitis C.

Dr. FAUCI. Mr. Honda, thank you for that question because it gives me the opportunity to say something that you do not get a chance to say very often. The past few years have really been representative of one of the more exciting breakthroughs in biomedical research, and that is the potential and real cure for hepatitis C.

We know hepatitis C is a very important problem, as you mentioned, 3 million cases per year. The leading cause of liver transplantation in the United States is hepatitis C. The treatment for hepatitis C prior to these breakthroughs was a long 48-week treatment with interferon alpha plus ribavirin, which is a very difficult drug to take for 48 weeks with a number of toxicities. The cure rate ranged from 35–40 to about 70 percent at the most. This disease disproportionately affects African Americans, particularly individuals with HIV infection.

Over the last few years, what are called direct acting agents, namely agents that are directly targeted against hepatitis C, have gone to the point with clinical trials—many of which have been involving the NIH, including the basic science—where we have moved to what we call interferon-free regimens using direct-acting agents. The recent data in a flurry of papers that came out in multiple journals, show that the cure rate even with the most difficult infections, type 1C and others, even in people with advanced disease, has gone up to anywhere between 85 and 95 percent.

Mr. HARRIS. Dr. Fauci, on that good news, I am going to cut you off. We could listen all day to the wonderful cures, and they really are, that are coming out. I recognize the vice chair, Mr. Womack.

Mr. WOMACK. Thank you very much, and thanks to the panel. I apologize. I was in another meeting, another hearing. We have several of these going on at the same time, so I am joining you late. I have gone from Warthogs in defense to warts, I guess, here. You have got to kind of re-shift your thinking.

[Laughter.]

Mr. WOMACK. Dr. Collins, my home State of Arkansas has a single academic health center, UAMS, as I am sure you are well aware. And it received a total of about \$47,000,000 and a half in NIH funding in Fiscal Year '13. That is less than half a percent of the research funding budget. You are aware of the huge impact these investments have on our economy, and in States with smaller populations and economies the impact is greatly amplified.

THE IDEA PROGRAM

The IDeA Program targets funding for merit-based, peer reviewed research and infrastructure grants at institutions in 23 States like mine that have historically received less NIH research support. Together in '13, the 23 States secured just 9 percent of NIH research funding. In your '14 budget, you proposed a \$51 million cut to the then \$276 million program. Ultimately it was appropriated at \$273 million level funding for '15.

So I would like to know, given the proposed cuts and request for level funding for the IDeA program, what the NIH is otherwise

doing to ensure that IDeA states remain competitive when competing for this kind of funding.

Dr. COLLINS. Thank you for the question. The IDeA program is very important to us, and as you pointed out, this is an opportunity to try to give talented scientists in States that do not have very high-powered research intensive universities the opportunity to provide those kinds of research experiences to train the next generation of scientists to do good work that is going to contribute substantially to the biomedical research enterprise.

I have visited a number of IDeA states. I have met with folks who are working there in those programs, and some of our centers of biomedical research excellence, the COBRE program. And I am impressed with what is able to go on there. I have not been to Arkansas, but maybe I should come by some time and see what you are doing.

Mr. WOMACK. Consider this an invitation.

Dr. COLLINS. I hear you. We are, of course, in a very stressed situation as far as our support for virtually everything. And so, certainly if the situation were more favorable, we would love to see the IDeA program also be a beneficiary of that. Since we have lost now more than 20 percent of our purchasing power for research in the last 10 years, everything has kind of been in a squeeze.

We are a meritocracy. We try to make sure that the funds that we put out there are for the very best science. I am delighted to say that quite a bit of that goes on in States like yours, and we want to encourage that. And we especially look to see sort of what is the success of that. Are we, in fact, encouraging people in the IDeA program to move forward, to get a successful R01 grant, to train an individual who goes on to become a leader in the field. And we are encouraged by what we see there, but we would always like to see it even stronger. It is a very important program for us.

Mr. WOMACK. Do you not sense that in a time when people are making decisions, family decisions, quality of life, looking for opportunities to go places to do this kind of research where there is a great quality of life and a lower economic impact on raising a family, putting them through college, buying the homes, and those kinds of things, does it not make sense that more and more of these types of individuals, these very brilliant people are going to make brilliant decisions about moving to great places like Arkansas?

[Laughter.]

Dr. COLLINS. You make a great case, Congressman. I think many of them probably, especially those who have financial constraints that would make it difficult to transition to a place that is much more costly in terms of cost of living, will choose to carry out their scientific programs in places that are not as expensive and perhaps closer to family. And we want to be sure if they do so, they have the chance to have the full-blown experience of what it takes to become a successful scientist.

Mr. WOMACK. One of the tough things that I have to address when I have disease-specific people coming to my office, and many of these people are not lobbyists, although they are there to lobby. They are people who have been hardshipped or affected personally by some of these particular issues. One of the hardest discussions

I ever have is to try to help them understand where those disease-specific issues are in the overall realm of the national priorities.

So help me—am I out of time already? If I have another round of questions, I will come back to that——

Mr. HARRIS. We will have another round.

Mr. WOMACK [continuing]. Line of thinking. But thank you very much.

PRIORITY SETTING AT NIH

Mr. HARRIS. So the first round of questions are done now, so I will begin the second round of questions. I am going to follow up on something the vice chair talked about and Mr. Joyce talked about.

You know, as the vice chairman mentions, we get a lot of people from advocacy groups coming to us, and Mr. Joyce asked, I guess, how do you prioritize, you know, where you are going to spend money on cross-diseases? And I will tell you, you certainly do not want the legislature involved. I will just tell you briefly.

In Maryland, when I first came to the State Senate in 1998, we had this mandate that you have to provide hospitalization after a mastectomy. Perfectly reasonable. But to make it politically correct, they then coupled it with saying you have to provide hospitalization after testicular cancer surgery. We are the only State in the Nation that has this. Political correctness is fine in some realms, but really science should be devoid of political correctness, and those decisions should be made devoid of that I think.

So I have got to ask, you know, we look over what the NIH spends, and I guess, Dr. Collins, you can agree that the legislature probably should not be telling you——

Dr. COLLINS. With you on that.

Mr. HARRIS. Okay. So I have a table of where we are spending the NIH money now and looking at various major diseases. And I am going to only concentrate on the ones where we have more than a million people affected, so where the prevalence is only a million people or more. And if you look at the research dollars, whether it is by the number of people with the disease per death from the disease, HIV/AIDS is 10 times the amount—10 times the amount—than, for instance, research per death from breast cancer, diabetes, research per person with 10 times the amount except pancreatic cancer where there are fewer people involved—breast cancer, stroke, cancers for all reasons.

I got to say, you look at this and you go, and we did great. Look, I was there, and most of you on the panel who are doctors, you know, started when we did not even know what caused it. I mean, it was this mystery disease. We have gone to pretty close to a cure, certainly with newborns transmission, and maybe, in fact, we may have a cure.

Why are we spending that much per person when we have other diseases that afflict more people? Why? And my understanding is there is a 10 percent, you know, earmark that exists. First of all, just to clarify, it is not statutory. This was not the legislature came in and said we are going to do this. So if it is not statutory, what is the origin? What is the history? Where are we going?

And when these groups come in, and I have these groups come in and they say, look, if you can just give us a 5 percent increase in next year's budget, it will make up for inflation. And you just say, maybe we should stop earmarking diseases because internally and then we all of a sudden have 10 percent more. What is the short answer? You do not have a whole lot of time. It is literally \$3,000,000,000.

Dr. COLLINS. So the answer needs to be a scientific one. I am going to turn to Dr. Fauci who oversees that research budget and have him respond.

Dr. FAUCI. So thank you for the question. I could understand the rationale for that question, but I think we need to remember that despite the fact that we have had great successes that you are very familiar with, and I need not take time to go through them, that we are still in the middle of a raging global pandemic. There are 36 million people living with HIV. There were 2.3 million new infections, 1.6 million deaths in 2012. In the United States, there have been 636,000 deaths, and every year we have 50,000 new infections.

Mr. HARRIS. Doctor, I am going to have to interrupt you. But you do not dispute that we spend about \$200,000 per death in the United States—

Dr. FAUCI. Right.

Mr. HARRIS [continuing]. On AIDS/HIV research. And we spend on stroke \$2,000 per death, on heart disease \$2,000 per death. These are tremendous problems in the United States. We get people in our offices all the time with all kinds of diseases. We are spending 10 times by any metric that is a reasonable metric.

If my constituents come to me and say how much are you spending per person if I have heart disease, we are spending 10 times the amount, 15 times the amount.

Dr. FAUCI. I understand, but if I can give—

Mr. HARRIS. Do you dispute those or do you think that is about the ballpark?

Dr. FAUCI. I do not dispute the numbers, Dr. Harris. But what I would like to throw on the table, whether you accept it or not, is that when you are dealing with an ongoing pandemic, it is different than a disease, as serious as it might be, that is essentially stable, that you want to bring down the death rate. We have the opportunity of eliminating this disease. That is the reason why we are putting so much money into it with regard to a vaccine and a cure.

Mr. HARRIS. Let me ask. That is fine. I mean, who decides on that 10 percent? I mean, it seems kind of arbitrary. I mean, you know, if we had imposed a mandate and said you have to spend 15 percent of your budget on heart disease, or 20 percent, or 25 percent, you would come back and say, how dare you. Believe me because I have this. How dare the legislature come and do this? You know, what is good for the goose ought to be good for the gander on this. Thank you very much.

Ms. DeLauro?

Ms. DELAURO. Thank you very much, Mr. Chairman. God help us if the legislature got involved in dictating to you what you should do. That has been really the hallmark of this wonderful,

wonderful subcommittee is that we do not do that. We take a look at what public health is about, global pandemics, et cetera, and where public health and safety arise.

I would like to just before moving onto a question here, this point has been made a couple of times, and I want to try to clear the air on it. This is about the evaluation taps. I think we should set the record straight here. Authorizing law sets the maximum evaluation transfer percentage at 1 percent.

This committee has been since that time overriding the authorizing law mandating a higher percentage. 2002 bill, percentage was raised from 1 percent to 1.2 percent, less than the increase to 2 percent proposed by the Bush Administration. 2006, percentage specified in the appropriations bill had been increased to 2.4 percent. Since then it has been increased by another 10th of a percent.

The perception here is that the transfers are the Secretary's decision. Not the case. She has limited discretion in this matter. The amount of the transfer, the specific uses of transferred funds, are spelled out in considerable detail in the annual appropriations bill. A small portion of the total is left unallocated by this committee. The transfers are this committee's doing, not the Secretary's doing. And anyone who does not read the language in the Consolidated 2014 Appropriations Act, which talks about the portion the Secretary determines, but not more than 2 and a half percent of any amounts appropriated for programs authorized under the act shall be made available for evaluation.

Now, if you have not read the document in terms of the budget summary where it says where it goes. It does not just disappear into the air. It says very clearly immunization and respiratory diseases, evaluation top funding \$12,864,000. We approved it. This committee does this. Get the facts. Read the summaries.

This committee said no to viral hepatitis, STD, and TB prevention where the evaluation tap was \$3,000,000. They said no. Understand evaluation taps, what it means. And I do not support going from 2 and a half percent to 3 percent. And I do support making up the shortfall of \$700,000,000. I wanted to do it in the omnibus bill, but we could not get agreement on setting that money. If we are so concerned about the NIH, then let's put our money where our mouths are in this effort when we have the opportunity to do it.

PHILANTHROPIC FUNDING OF MEDICAL RESEARCH

So thank you very much, Mr. Chairman. If I can, let me try to get in a question on the issue of private philanthropic funding of medical research. It is expensive, but we are looking at contributions from wealthy individuals to support medical research. I applaud the individual commitment, I really do. I think it is terrific. But it is finding cures to diseases that affected either themselves or people who are close to them. Cancer survivor, as I am, life-threatening disease, it inspires you to get involved in what you are doing.

The New York Times had an article that says that privately funding research could be a replacement for publicly funded research. I laud the private effort. I welcome it. I do not believe our

decisions on basic research should be outsourced to the private sector.

What is the right balance, public, private investment in biomedical research? How can we ensure that the national priorities are remaining the guiding influence in biomedical research? And let's go, and I will get back to it if I have a chance of doing it. This is what I think is fundamentally important about our future. Dr. Collins?

Dr. COLLINS. Well, I very much appreciate your question. The article by Bill Broad in *The New York Times* laid out in great detail the amount of philanthropy that is now being focused on biomedical. And we are grateful to see that kind of contribution to research in order to go faster.

But you are quite right. It could in no way substitute for the major support of biomedical research in the United States, which is the National Institutes of Health. It could not substitute in quantity because if you add up all of the philanthropy, it is still a small percentage of what NIH supports, nor could it manage to survey the entire landscape to identify where the needs are the greatest and make sure that the distribution of funds is happening in a scientifically balanced way. Just to say, as I say, we welcome those kinds of partnerships. We would love to see more of them.

There is a very interesting op-ed in *The New York Times* today responding to that, suggesting that one should be thoughtful. Where is the greatest entrepreneur in the American biomedical research enterprise? It is the Federal government. You can see it over and over and over again.

Ms. DELAURO. Thank you. I would love to pursue this conversation with all of you individually offline. I think it is a critically important question for our future and where we go, and I am sure you are all discussing it internally. I would love to have the opportunity to talk with you about it.

Mr. HARRIS. Thank you very much. Mr. Womack.

INNOVATIVE VERSUS DISCOVERY

Mr. WOMACK. Second round is going good. Historically, NIH is a discovery organization. However, it has focused more in the past 5 years on innovation. Is there a clear role at NIH for innovating? And I would like for you to explain the difference between innovation and discovery and how the NIH balances between the two priorities.

Dr. COLLINS. I love the question, and I would say innovation and discovery are not mutually exclusive. There is a lot of innovation that can be introduced into discovery science where you are trying to find out what is the fundamental basis, for instance, of how the brain works. Well, that is a discovery, but, boy, it is going to take a lot of innovation in terms of new technologies to be able to actually understand how those 85 billion neurons between our ears are functioning in remarkably complex ways, building circuits that can do things that are really almost impossible to imagine in terms of laying down memories and retrieving them and all that. So that is innovation, but it is also discovery.

We are all about innovation, recognizing that, in fact, our real engine of innovation in many instances is the best and brightest

minds out there in this country in our finest institutions who are coming up with ideas that we probably in a top down way could not have come up with. And so, a big part of our portfolio is these so-called investigator initiated grants that come to us, that go through the most rigorous peer review system that get prioritized based on their promise, and then funded these days if they happen to be in the top 16 percent. And we are throwing away a lot of innovation because that success rate has fallen so low.

We are doing other things to try to specifically encourage innovation thinking with programs like the Pioneer Award. The Pioneer Award, which has now been in place for 10 years, encourages investigators to come to us with ideas that are pretty out of the box, and which they do not have a lot of preliminary data or to prove that it is going to work. They have to be able to show that if it does work, it is going to be groundbreaking. That program has produced a lot of very exciting science, including just today in my blog, which I am sure all of you read.

[Laughter.]

Dr. COLLINS. I wrote about a new discovery in Alzheimer's disease, a master switch called REST, which was discovered by a pioneer awardee, which is a whole new insight into what is happening in the Alzheimer's brain that came completely by surprise. Now, that was innovative, but we gave the investigator the chance to do that.

We are considering at NIH expanding that kind of program, giving more of our portfolio in a way that the Institutes are going to experiment with to individuals who have out of the box ideas rather than expecting them to write a very detailed project-based kind of proposal to us.

In addition to that, we have large-scale enterprises focused on specific areas that no single investigator could do, but a team could. The Genome Project was perhaps the most notable example, but we have many others—the MCODE Project, the Microbiome Project, and this BRAIN Initiative, which aims to bring together disciplines that have not necessarily worked well with each other, get them to know each other and do something significant.

STRATEGIC PLANNING AT NIH

Mr. WOMACK. Last question for you, and we can talk about these kinds of issues forever, but I want to go back to my priority question. I have got people in my office. They are suffering from these disease specific—why do we not do more pancreatic cancer, the high mortality rate, et cetera, et cetera? How do we establish these priorities? And I agree with my friend Ms. DeLauro that Congress should not be dictating what these are. We are not qualified to do that. So how do we discern?

Dr. COLLINS. In the report language for the Fiscal Year '14 appropriation, we are asked to put forward, and we will do so, how we manage the strategic planning process. All 27 of the Institutes, four of them represented here, have this process where they survey the landscape, they look at their portfolio, they try to see what other organizations are doing. They try to identify gaps and identify opportunities, and then make the decisions about how to distribute the funds that they have direct control over in that fashion,

but leaving a substantial amount of the dollars for those unanticipated ideas that are going to come to us from community that we want to be sure we are supporting as well.

It is a very carefully planned complex process. You will see a more detailed report about it later this year, but I think it is the right way to take the scientific and the public health needs and factor them into an outcome that does the best with the dollars we are given.

Mr. WOMACK. Let's give Dr. Varmus a real quick chance.

Dr. VARMUS. Just a couple of quick comments. Some of our own investigators fail to recognize the power of what we call program announcements. So if the NIH and the NIH Institutes want to emphasize a certain area without setting money aside, they can say, "We are particularly interested. Send us your best ideas, and we will fund them." And that is a very good way for us to exercise both our judgment about what needs to be done and to bring forth the best applications.

One point about innovation. I would strongly suggest that we get greater clarity about the meaning of the word. Innovation in my lexicon does not mean simply particularly imaginative or exciting work. It means something that is applied, and I think that you may have been meaning it in that sense as well. And I think it is a legitimate discussion to think about how much we should be spending on application as opposed to fundamental basic discovery.

Mr. HARRIS. Thank you very much. Ms. Lee?

NATIONAL INSTITUTE ON MINORITY HEALTH AND HEALTH DISPARITIES

Ms. LEE. Thank you. Okay. Once again, let me ask Dr. Collins about the National Institute on Minority Health and Health Disparities. Health disparities in the African-American, Latino, and Asian-Pacific American community are huge. And you know what they are: life expectancy much shorter, hypertension, diabetes, hepatitis. All of the diseases are disproportionate in communities of color. So that is one of the missions of the Institute, yet the funding does not seem to follow the huge job that we have asked you to do.

So I just wanted to ask you in terms of the budget, given that the Institute has a much smaller budget, I would have wanted to have seen a much larger request. So can you kind of explain what that is about? And also how the decision was made to not propose an increase in funding for this very important Institute.

Dr. COLLINS. Congresswoman Lee, thank you for the question. I will tell you, health disparities is, from my perspective, one of our highest priorities, and I would want to explain the answer to the question that you have posed.

Let's be clear. The health disparities at NIH is conducted by virtually all of the Institutes. The National Institute on Minority Health and Health Disparities (NIMHD) acts as a hub to build collaborations and to stimulate the kind of projects that need to happen. But most of the money we spend on the health disparities result is actually spent by the other Institutes in collaboration with NIMHD, and you would want that because that is where most of the budget resides.

In terms of what has happened with budget proposals going from '14 to '15, I would ask you not to make a great deal out of the small

differences in terms of what has happened with individual Institutes and Centers. Much of this was built upon specific initiatives, like the BRAIN project, that needed to have a ramp up in order to try to get onto a trajectory where something could happen. And so, institutes that happen to have larger parts of that, like my colleague to my right here of the "Neurology Institute," saw an increment that looks more promising, but, in fact, in reality the sort of base of full-blown activities that go on across NIH, including in NIMHD, there have not been winners and losers.

Frankly, everybody is pretty stressed as you can imagine given the fact that we, even with the Fiscal Year '15 proposal, we will still be a billion dollars short of where we were in Fiscal Year '12, as has already been pointed out.

I want to point out something else that I failed to mention when you asked earlier about our research workforce, and that is the recruitment of a chief officer for scientific workforce development, a new position that I have created. And this is Dr. Hannah Valentine, a highly regarded cardiovascular physician from Stanford who will actually be sworn in by me next Monday as a person to take on this role, emphasizing again just how seriously we are taking this opportunity.

I might also mention Dr. John Ruffin has just announced his intention to retire after 24 years of leading Minority Health and Health Disparities research, and we will be announcing fairly soon a very impressive search committee to go out and do a remarkable, strong national search to find a replacement for somebody to lead that critical part of NIH.

Ms. LEE. Well, Dr. Ruffin has certainly done a phenomenal job, and so give him my regards and my thanks.

I guess following up from what you just indicated, my concern then is, with the Institute being the major coordinating function, still without an increase, how does this funding really enhance the coordination that this Institute, the National Institute on Minority Health and Health Disparities (NIMHD), has because that is a major, major job?

Dr. COLLINS. It is, and they are not unique in taking on that kind of role in terms of being a hub for broader activity. Look at our new National Center for Advancing Translational Sciences (NCATS). And everybody would say translation is really important right now. The budget for NCATS is actually quite modest. But the ability to build those collaborations, the impact is much greater. Look at the Genome Institute where I used to be. Everybody is doing genomics now, but the "Genome Institute" is a small fraction of that total.

NIMHD has the opportunity with their position in the scheme of things to encourage, to recruit participation in a wide variety of programs. Maybe I will just quickly ask Dr. Gibbons, as somebody who has a lot of health disparity research going on in his Institute, how does that work?

Dr. GIBBONS. Well, certainly for our cohort studies, we have a very diverse portfolio that is very inclusive and reflective of America—the Strong Heart study of Native Americans, the Jackson Heart study that I am sure you are familiar with, and African Americans, the Hispanic Community Health study, the largest

study of Latino health with 15,000 participants, the Mesa study, the Multi-Ethnic Study of Atherosclerosis. These are studies that we are doing to address minority health many times in collaboration and in close coordination with the NIMHD.

Mr. HARRIS. Thank you very much, Dr. Gibbons.

Ms. Roby, thank you for your patience. You are up.

Ms. ROBY. Thanks. Thank you again, all, for being here today. I have learned a lot, and being a new member of this committee it has been really great to sit here and get to hear, you know, specifically what you do and what you are contributing to so many wonderful things. And I look forward to many more success stories.

And so, we have a plethora of disease-specific questions that we are going to submit to you for the record. You know, as Mr. Womack pointed out, this is the time of year when a lot of these folks come to our offices and come not with D.C. lobbyists, but come, you know, as a survivor of a disease or someone who is living with a disease. And it is very helpful to be able to look them in the eye and report back to them about, you know, what advances are being made, but also what challenges are there and how to better advocate for their disease. So if you will, when we submit this to you, if we could get a prompt response that would be great.

Several other members have touched on the requirements in the omnibus that you provide with this report within 180 days. And I know this has come up, but I just wanted to give you one opportunity again before the end of this hearing if you wanted to share with this committee any specific or relevant updates as it relates to the process for internal controls that you are going through to help mitigate the possibility for duplication across the ICs. If there is anything else you want to share with us before we conclude this hearing.

Dr. COLLINS. You know, I have talked in a general way about how the 27 Institutes do this. I am going to ask Dr. Fauci to say in specific ways what has happened with NIAID as a place that has been very careful about a strategic plan for their own research efforts. Tony, can you say a word?

Dr. FAUCI. We have a strategic plan that we update intermittently in which we get input from outside investigators, have program meetings, both policy and programmatic meetings, and end up with a coordinated plan that is available for everyone to see.

I just happen to have for you here an example—you like things that hang around, so this is our strategic plan for NIAID for 2013. And virtually every Institute does that, and as we all meet very frequently, it essentially gets into one group so that we have a very good idea about what we are doing and what the planning situation is.

Ms. ROBY. And that helps prevent that overlap.

Dr. FAUCI. Oh, yes, but indeed I think we need to point out, as I am sure any of my colleagues would say, that when you talk about overlap, you want to make sure you distinguish the synergy of things that you do from things that are unnecessary and non-productive duplication.

Ms. ROBY. Some of it is necessary.

Dr. FAUCI. Yes, it really is. In fact, the fact is that the biological disciplines interdigitate an awful lot. For example, and, in fact, you

just heard that when Harold Varmus and I answered essentially the same question. You have a virus that causes cancer. You have an Institute with extraordinary expertise in cancer and an institute with extraordinary expertise in infectious diseases. We work together an extraordinary amount, not only with hepatitis B, hepatitis C, human papillomavirus, EB virus, and all of those things, HIV all the time because of the co-morbidities with cancer.

It is really something that when you really dig deep into it, you see an extraordinary amount of synergy as opposed to duplication and overlap.

Ms. ROBY. Great. I yield back.

YOUNG INVESTIGATORS

Mr. HARRIS. Thank you very much. And we have enough time for one more quick round because you are riveting us. You would like not to be so riveted, huh?

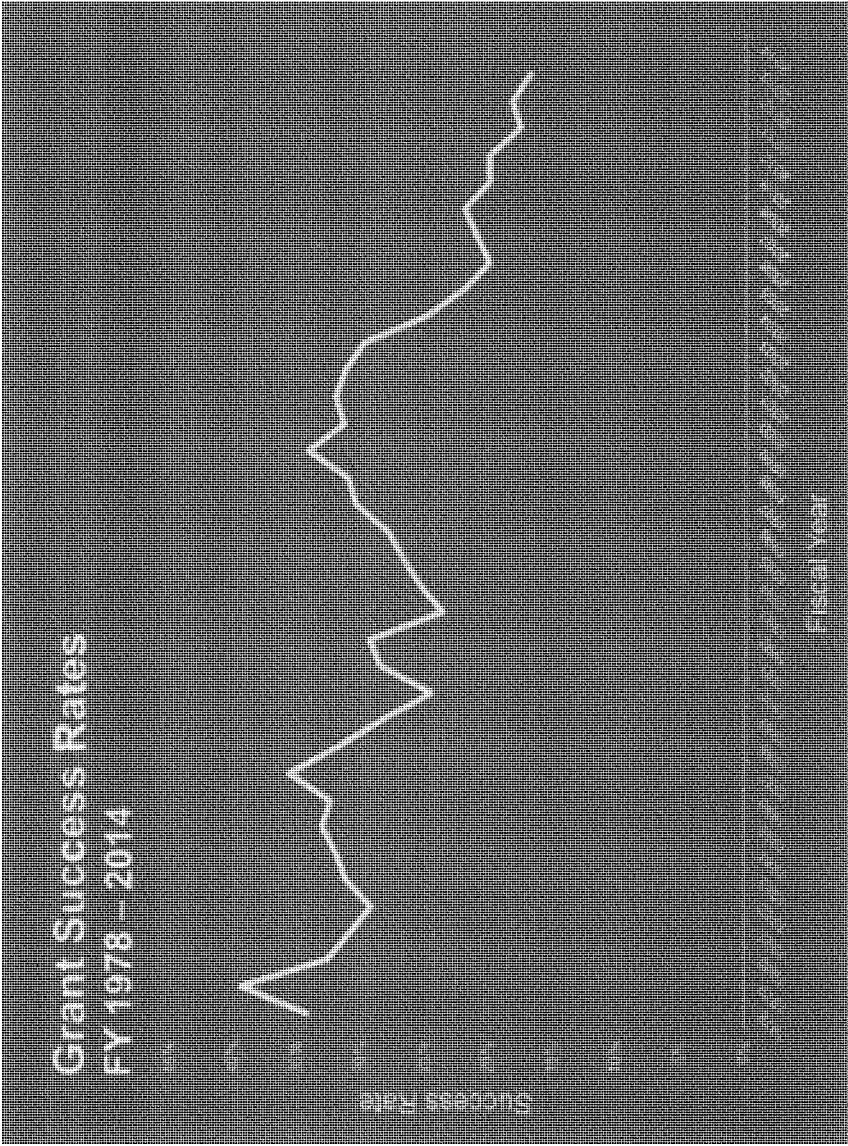
Thank you. Again, thank all of you for the work you do. I am going to ask you, Dr. Collins, to respond. I think there was an op-ed was about a month and a half ago in the Wall Street Journal written by Doctors Daniel and Rothman about young investigators and funding. And you know it is one of the roles that I believe the NIH has to have because there is no one else who is going to do it, is make sure that we have young investigators.

Can you confirm the data in the article, I mean, because it always brought to our attention, well, you know, when you level off funding, the trouble is that, you know, you cannot fund this many young investigators you otherwise would. But the article suggested that the number of young investigators actually has gone down, so it has not leveled off. Actually the distribution has now become where there are more senior, less young investigators, a trend that would be very disturbing to me because I actually think an increasing percent of the NIH budget, not a decreasing percent, ought to be going to these young, promising investigators.

So if you could just briefly say, how do you envision that we can turn the tide on that? I mean, the trend line is not good. I personally would like to see it reversed as soon as possible, and to do that, you have got to do something differently than you do now.

Dr. COLLINS. Well, I appreciate the question. Part of the problem as shown on this graph, which shows you the overall challenge that we face over the last 30 or 40 years with the ability of an investigator to get funded. That is the chance of being successfully funded if you sent your best idea to NIH, and which has now reached a historic low of 16.8 percent.

[The information follows:]



Dr. COLLINS. That of course, does not just affect young investigators. That affects everybody. But it is perhaps particularly challenging for a young investigator faced with that to decide whether they can get their efforts going or not. It has resulted in many young investigators staying in post-doctoral training period for much longer than is probably good for them. The average age at first grant award has crept up to about age 42, which is clearly not healthy.

We do various things at NIH to try to encourage young investigators to be successful. One is what is called a K-99 award, which gives a post-doctoral fellow a chance to apply for their own support, and if they are successful, it bridges them over to an academic position. So they arrive at an academic institution already supported for 2 or 3 years. That is a very nice way to market yourself if you are looking for a faculty position, and that has been a good program.

We have just started, and this is one of my own programs that I am watching very closely, a program to allow individuals who have just obtained a Ph.D. and are particularly innovative and independent to go straight on to a faculty position. It is the Skip the Post-Doc Award, because sometimes people are not really needing that. We fund only a few of those, about 15 each year. I go to their presentations. They are astounding.

Mr. HARRIS. But, Dr. Collins, with 15 a year, it will take you decades to reverse the problem. How fast can we reverse that problem, because, again, that trend is very, very disturbing to me.

Dr. COLLINS. Well, frankly, Dr. Harris, until we are able to do something about the overall problem of such a very difficult support system—

Mr. HARRIS. Dr. Collins—

Dr. COLLINS [continuing]. It is going to be hard to have a metric.

Mr. HARRIS [continuing]. That is only if you do not treat young investigators differently and somehow get them out of that pool.

Dr. COLLINS. Well, one thing we do, and I should have said this, is young investigators compete against each other. They do not compete against the established investigators, which helps quite a bit in terms of their being able to get on the on ramp to being supported, because otherwise they would do less well than they currently do. That helps a bit, but—

Mr. HARRIS. The numbers just do not show it. You have got to do something a little bit more. Dr. Landis—

Dr. LANDIS. If I could say, many institutes, including my own, have a special pay program for early-stage investigators where we pay them past the normal pay line for the general pool of investigators, and we have a significant number every council round. I look at each of those applications. Are they truly independent? Is the idea meritorious? And we fund them out of order, and we have seen a significant increase in our pool of early stage investigators. The problem is when they come in for their first competing renewal.

Mr. HARRIS. Sure. No, I understand. Dr. Fauci?

Dr. FAUCI. So just to add to Story's last point, we also, and I think most every Institute has a differential pay line for new and young investigators. So they operate, as Francis said, almost competing against each other.

Mr. HARRIS. I know, but it is not working.

Dr. FAUCI. I know, but——

Mr. HARRIS. I just want to emphasize, whatever you are doing, and I applaud it because it might even be worse if you were not doing what you were doing.

Dr. COLLINS. It would be.

Mr. HARRIS. But it has got to get better. And my time is up, and I would be more than happy to make a visit and discuss this with you at length, you know, on the campus. This is of great value to me.

Ms. DeLauro?

Ms. DELAURO. Thank you, Mr. Chairman.

Mr. HARRIS. Your turn to be passionate.

Ms. DELAURO. And accurate, I might add, because in this regard——

Mr. HARRIS. Ouch.

Ms. DELAURO. Yes.

[Laughter.]

Ms. DELAURO. I think that what you have been doing, and we did not talk about the Kirschstein Research Awards. There are training stipends, and they have ranges. The post-doc award is \$42,000 to \$55,000 a year, depending on your years of experience. It falls short of a goal set in 2001 for the entry-level stipend to start at \$45,000. The NIH funding, I note in this budget, would be about 2 percent. It is an increase in the President's budget.

But if we are serious about pipelines, and new young researchers and their success rate, and we put a very, very big emphasis on this, then we need to be serious about the kinds of allocations that this subcommittee gets so that, in fact, it can place the resources where the priorities are. And if we continue to put our heads in the sand with regard to investment, whether it be in training or whether it be in biomedical research or in other areas, then we move backward instead of forward.

I have a couple of quick questions because I can follow up. Dr. Landis, your study, male/female brain?

MALE/FEMALE BRAIN

Dr. LANDIS. So the——

Ms. DELAURO. You got to be fast. He will cut me off.

Dr. LANDIS. The BRAIN Initiative is focused primarily on creating tools and technologies to allow us to understand the brain. Let me give you an example of a study that is using current technologies. It is the Human Brain Connectome, producing connectome maps of 300 different young adults equally balanced, male and female.

Ms. DELAURO. Male and female, okay.

Dr. LANDIS. Those data will be publicly available. And one of the questions that will be answered in those data is what are the differences between males and females.

Ms. DELAURO. Thank you. Thank you because Alzheimer's does affect women more so than it does men. We need to find out.

Dr. Fauci, you talked about vaccinations with regard to Mrs. Lowey before. You talked about measles. Just a quick question on the cervical cancer vaccine. Should we have more education on the

use of that with regard to human papillomavirus and cervical cancer?

Dr. FAUCI. The answer is yes, and I will turn it over to Dr. Varmus also. But the principle is a very important principle. When you have a vaccine that really works, it is important to educate people to get the vaccine.

Ms. DELAURO. And you have to avoid and respond to misinformation. Dr. Varmus?

Dr. VARMUS. Just very briefly, I would like to draw the committee's attention to a recent report from the President's Cancer Panel, which works in conjunction with the NCI, that strongly recommends a number of measures we think would improve the unfortunately low vaccination rates both for males—

Ms. DELAURO. And females. Bingo.

Dr. VARMUS. Males are important here. It is a forgotten subspecies in the use of this vaccine—there is an increased rate of oropharyngeal cancer caused by a strain of the virus that is covered by the vaccine, and we need to vaccinate males as well as females.

Ms. DELAURO. And we have to move out of misinformation and philosophical interpretation of these efforts. I am going to send the NCI and funding and research, et cetera.

I must tell you, you talked about cancer immunotherapy, which is extraordinary. It really is extraordinary. The tissue chip we did not spend much time on, but I am going to just tell you that this morning at an earlier meeting, I am going to tell you, the Humane Society, over the top about this effort and wanted you to know that you have got people that are, you know, doves are supportive of this tissue chip effort. The Accelerating Medicines Partnership, (AMP), a second on that. Tell me where—

THE ACCELERATING MEDICINES PARTNERSHIP

Dr. COLLINS. Thanks for that question. This is a very exciting new initiative, NIH partnering with 10 pharmaceutical companies to take this deluge of new discoveries about the molecular basis of disease and move them forward to generate the next generation of drugs by identifying what are the drug targets that have not previously been chased after that are most likely to lead to success.

Ms. DELAURO. Drug targets.

Dr. COLLINS. You want to identify, and once you understand the pathway that seems to go awry, say, in diabetes, which part of that pathway could actually be interfered with in a positive way by a drug? That would be the drug target to go after. AMP is going after diabetes, Alzheimer's disease, rheumatoid arthritis, and lupus, and that is just for starters. We would like to see that list expand. It is just getting under way. NIH and pharma are paying 50/50 to make this go, scientists sitting around the same table and all open access to the data.

Ms. DELAURO. I will put questions in on asthma and allergy research, and also on the issue of prescription drug abuse, but I will get those for the record.

Mr. HARRIS. Thank you very much. Ms. Lee?

Ms. DELAURO. God bless you.

SICKLE CELL RESEARCH

Ms. LEE. Thank you very much. Dr. Collins, let me ask you a couple of things about sickle cell research. Of course, this sub-committee, and thank you very much for your work with regard to the whole issue of sickle trait and diabetes and the A1C test. I just wanted to know where we are. For those of you who have not been following this, we learned, I learned, and this was through talking and doing my own personal research, that the A1C test could give a misdiagnosis if one has the sickle cell trait. And the NIH and your institute, I think it was for diabetes, digestive, and kidney disease, really helped mount a public awareness campaign.

I want to make sure now that the doctors and labs know not to give that A1C test, but to check first for the sickle cell trait. And so, it is kind of a catch-22. So I want to know how that is going, and are we seeing a change now and a shift. Secondly, just what additional treatments for sickle cell disease have been identified and pursued, and how the state of the research is. And then thirdly, how in the world do people really know they have the sickle cell trait?

I mean, I know when babies are born, I believe they are tested. But when you turn 18 or 17 or 15 or 21 or 30, do you get retested again? Are you encouraged if you have diabetes to get tested for the sickle cell trait? I mean, how do we really address this because it is very serious in communities with Mediterranean descent, African-American communities, communities of color, and its something that we have not really gotten our hands around yet.

Dr. COLLINS. So I will start, and then I may ask Dr. Gibbons to weigh in a little bit since sickle cell disease is in the Heart, Lung, and Blood Institute's portfolio.

With regard to your concern, though about the diabetes overlap, that is something that Dr. Rogers, who is the director of NIDDK, has been tracking. And I actually anticipated you might ask about this and spoke to him this morning. I think it is quite encouraging that thanks to the awareness of this, and you have helped with that, there are now tests that are the ones that are generally now used that do not have this problem of giving you a false positive for hemoglobin A1C because the person happened to have sickle cell trait. That was the difficulty. People were being given wrong information about diabetes because of the overlap between these different types of hemoglobin.

And now, it does seem that that is generally recognized in most places. Of course, given our medical care system, I cannot tell you it is 100 percent, but I think a lot of awareness has happened, and the test that is now generally used does not have this problem. So we have made a lot of progress there.

In terms of new therapies, goodness, a lot of excitement in sickle cell disease, and well there should be after all these years of trying to come up with an effective strategy going beyond hydroxyurea. There is a phase-two trial going on that was actually started with in the National Center for Advancing Translational Sciences, NCATS, as a collaboration with a company in Boston that actually is a compound that keeps the sickle hemoglobin from cycling. And it is looking pretty exciting. It turned out to be very safe, and in

a very small study seemed to be showing some benefit in terms of oxygenation.

And there is a whole host of new ways to try to understand how to turn fetal hemoglobin back on because that can be protective for people who have sickle cell disease or thalassemia. And the discoveries coming out of genomics are teaching us about something called, of all things, BCL11A, which turns out to be a great new drug target that you never would have guessed with all the right properties for this disease.

Dr. GIBBONS, please fill in there in terms of how do people—

Ms. LEE. And, Dr. Gibbons, yes, can you let us know how people know they have the trait or what is required now so people will know their status?

Dr. GIBBONS. Certainly a great boon has been the newborn screening efforts that have helped to identify individuals with sickle cell disease. Certainly we still need to do more research in terms of sickle cell trait, and recognizing that that reflects their ancestry in those endemic areas of malaria, and that there are associations with other complications that can occur with traits.

We are learning more and more about that. Indeed, there is an interaction that is recently being identified between those with sickle cell trait and another genetic variant that relates to predisposition to chronic kidney disease.

Ms. LEE. But let me ask you, though, at 50 years old, how does one know they have the sickle cell trait?

Dr. GIBBONS. Well, certainly as Dr. Collins mentioned, there are diagnostic tests.

Ms. LEE. Well, how do physicians determine to look for that?

Dr. GIBBONS. So as you point out, I think part of the key is awareness and education to both patients and providers as they understand the significance of the trait and us doing more research to indicate why it is important for that to be identified and the implications, and then disseminating that information more effectively to patients and providers. That is certainly something we need to do more of.

Ms. LEE. We need to do more, and I would like to follow up with you because that is still a little too loose for me.

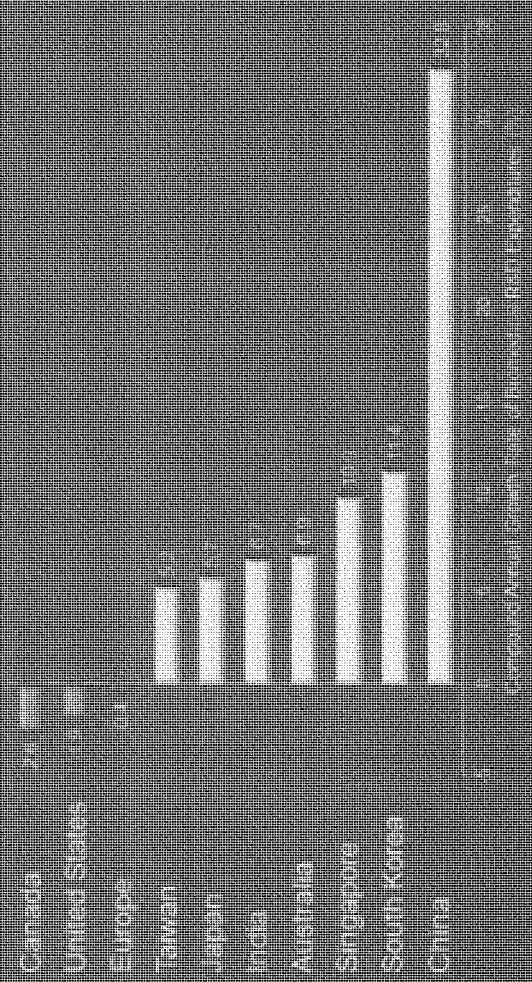
Mr. HARRIS. Thank you very much. We are going to gavel the session to a close in just a minute. But, Ms. DeLauro, would you like to make a closing comment?

Ms. DELAURO. Sure. Thank you very much, Mr. Chairman. You are all aware, and this was something that my colleague, Mrs. Lowey, talked about in her opening remarks, and that is the issue of global competitiveness. And I know you are aware our global R&D funding forecast was published in 2011, showed a decline in U.S. research commitment relative to our GDP, as other nations have moved progressively forward to increase their investments in both research and in life science.

The New England Journal of Medicine really confirmed this trend. From 2007 to 2012, countries' average annual investment in biomedical R&D increased 33 percent in China, 12 percent in South Korea, 10 percent in Singapore, and it fell by 2 percent in the United States.

Dr. COLLINS. I just happen to have that graph, and I have just put it up there that you were describing the figures.
[The information follows:]

Compound Annual Growth Rate of Biomedical R&D Expenditures by Country, Adjusted for Inflation 2007–2012



Source: N Engl J Med. 2013;Jan 2;359(1):3-6

Ms. DELAURO. Our share, U.S. share, of biomedical R&D expenditure declined from 52 percent to 42 percent despite what is happening in the rest of the world. China's biomedical R&D expenditure grew 313 percent during that period of time. I know it is of concern to you. It needs to be of concern to us if we are to be at the cutting edge of discovery as you are.

And if it is not about the humanity of what you do and its benefits, then about the economic benefits of what you do. That has to be of singular concern and of singular priority as we move forward. You are moving forward. This subcommittee needs to move forward in making sure that we are strengthening our R&D capability in every area, but particularly, with that gift of life that you provide.

Thank you very, very much for being here this morning.

Mr. HARRIS. Thank you very much. And just before I close, you know, again, I appreciate that China is increasing their investment a tremendous percent, but let's remember that their public investment in that New England Journal article is \$2 billion. That is it, the tap plus the decrease in funding to advance science that the Administration in their budget would equal the entire public funding of research in China.

You know, and because the accuracy was questioned, I am just going to include in the record that the article that I referenced from the Wall Street Journal indicated between 1980 and 2012 when the number of grants that the NIH funded doubled, the number of grants issued to investigators under 35 decreased by 40 percent, reflected and mirrored in the R01 experience as well.

Mr. HARRIS. So I would urge you, again, I think this is a very important topic. You have got to do something to reverse that. I mean, I was a young investigator. I had young investigators work for me. I would urge you to do that.

And with that, I am just going to remind the members and the panel that we are going to keep the record open for 2 weeks for additional questions to be submitted.

Mr. HARRIS. And I want to thank all of you for the work you do and taking out the time. And I am going to agree with the ranking member. You know, I think it was the ranking member of the committee who had said it is probably best if you all just get back to your work and help America's health, and spend as little as possible here on the Hill.

Thank you very much.

Dr. COLLINS. We are on our way.

**Department of Labor, Health and Human Services and Education and Related
Agencies
NIH Budget Hearing – Future of Biomedical Research**

March 26, 2014

Questions for the Record – Chairman Kingston

1) Questionable Research Grants

Dr. Collins, I want to follow-up on my opening statement comments related to “questionable grants” and the activity related to this priority setting review.

- a) Can you share with us what changes NIH is considering as it conducts this review to improve accountability in the funding process?
- b) Is NIH working with any outside organizations like the National Academy of Public Administration or others to develop a more systematic and strategic approach to bio-medical research linked to the NIH core mission, in lieu funding grants related to an Experimental Design of a Social Security System in the Yucatan or grants related to Public Health Education and Campaigns in China?

Answer:

As instructed in the explanatory statement accompanying the Consolidated Appropriations Act, 2014 (P.L. 113-76) and under the authority of the responsibilities outlined in the Public Health Service Act, the NIH Director is actively engaged in an NIH-wide priority setting review to examine whether and how decision makers at NIH have access to the information they need to make key priority setting and resource allocation decisions. As part of this review, each IC and several OD offices have provided a description of their priority setting and funding decision making processes, which will be compiled and analyzed to determine strengths and potential weaknesses of NIH’s current processes. While NIH strives to always use its public funds responsibly and effectively, refining the decision making process by allocating additional resources to guide priority setting may improve accountability in the funding process.

While the NIH-wide priority setting review is still underway, preliminary findings indicate that NIH’s priority setting process includes the necessary rigorous evaluation of a project’s scientific merit, potential contribution to public health, fit within the portfolio, and assessment of whether it capitalizes on scientific opportunities in the field or fills a research gap. This systematic process includes receiving valuable input from numerous stakeholders to ensure that NIH is responsive to ongoing and emerging public health needs. Prior to funding, all NIH research projects are assessed in relation to the IC’s and NIH’s missions. Thus, all NIH-funded research supports the core mission of the agency. Although some grants may appear to be outside the scope of NIH’s mission at first glance, the scientific importance of the research is verified through multiple levels of review.

The NIH understands that it must demonstrate that it is making the best use of taxpayer dollars to pursue science that contributes to public health. While the NIH is not working with the National Academy of Public Administration, the agency is engaging in activities to ensure that the highest quality research to advance science and improve health is funded. For example, the Office of Portfolio Analysis (OPA) within the Division of Program Coordination, Planning, and Strategic Initiatives in the Office of the Director is responsible for advancing NIH efforts to improve decision-making through data-driven approaches and has a well-established role in developing new computational tools and methods. These new approaches represent important advances in our ability to measure the output of NIH investments, track the translation of basic scientific knowledge into advances in human health, identify areas of opportunity, and highlight potential overlap. OPA continues to expand its existing training program, resources, and class offerings available to NIH staff. OPA tools can mine information in NIH grant applications, publications, or patents, to enable effective and efficient management of the NIH research portfolio.

As part of its efforts to steward public funds appropriately, NIH also relies on the advice of numerous external advisors to inform the agency's strategic planning and priority setting activities. Groups such as the National Academies of Science may evaluate an NIH program and recommend adjusting research priorities going forward, and other advisory groups may take an inventory of Federal activities in a particular research area to identify research gaps, potential partnerships, and scientific opportunities. All of these activities contribute to the corpus of knowledge used by decision makers at NIH to set priorities and make funding decisions that keep NIH research focused on its core mission to seek fundamental knowledge about the nature and behavior of living systems and to apply that knowledge to enhance health, lengthen life, and reduce illness and disability.

NIH looks forward to providing Congress with the full report on its priority setting review in the near future.

2) Allocation of Funds

The NIH mission is to invest in basic biomedical research to uncover new knowledge that can lead to better health and disease cures for all Americans. I assume that while NIH tries to balance the need for rare and neglected disease, it also focuses on diseases that have the largest health burden to the American population.

I understand that in 2011, the CDC reported the leading cause of death was Heart Disease with almost 600,000 deaths and according to the NIH Research, Conditions, and Disease Categories (RCDC). In FY 2012, NIH spent almost \$1.3 billion on this disease, which was less than 1% of the RCDC total amount or 4% of the NIH appropriated level.

Diabetes, which CDC listed as the 7th leading cause of death that killed over 73,000 Americans in 2011 – RCDC reported just over a billion, again less than 1% of the RCDC level or over 3% of the NIH appropriated level.

Although, HIV/AIDS which is not listed in CDC's top 15 leading causes of deaths in the US during 2011 was funded at over \$3 billion, which is more than 2% of the RCDC total and 10 percent of the NIH appropriation level.

I am not making a value judgment of one disease or leading cause of death over another. It is hard for me or the public to understand how NIH determines its priorities for the funding allocation proposed in the budget or how it measures sustained progress towards scientific research objectives.

- A) Frankly, it appears to be a random walk. Please explain what the NIH-wide funding criteria are during the development of the budget request and during the year of execution?

Answer:

Setting research priorities for NIH is based on a dynamic balance between public health needs, scientific opportunities, portfolio balance, and budgetary considerations. According to two recent academic analyses, on the whole, NIH funding levels for specific diseases align well with various measures of burden of disease, including deaths, hospitalizations, years of life lost (YLL), and Disability Adjusted Life Years (DALYs).^{1,2}

Four principles govern how NIH sets its research priorities:

- **Peer Review:** NIH only funds research which has been judged highly meritorious through a two-stage peer review process. The first level of review is for scientific merit and the second level of review, which includes public health relevance and portfolio balance considerations among other factors, is performed by the National Advisory Councils or Boards of each of the NIH Institutes and Centers (ICs).
- **Scientific Opportunities:** Significant research advances occur when new findings, often completely unexpected, open up new experimental possibilities and pathways. NIH constantly assesses its research portfolio in light of the latest scientific developments. Not all disease or scientific problems are equally ripe for new advances, nor do such advances come at the same rate across the portfolio.
- **Public Health Needs:** NIH responds to public health needs, ranging from emerging infectious diseases to the growing burden of chronic diseases. Rare diseases also affect the public's health, but these illnesses may not have the same funding incentives as more prevalent disorders, making it less likely that private funders will support rare disease research. NIH fills this research gap by funding studies on such diseases.
- **Portfolio Balance:** We cannot always predict the next public health emergency or anticipate the next scientific revelation that will arise. NIH strives to achieve a balanced portfolio within the overall framework of our mission, spreading our investments out to cover a mix of basic to applied, cellular to whole animal, and animal to human research, among many other factors. Substantial investments are

¹ Gillum L.A., Gouveia C., Dorsey E.R., Pletcher M., Mathers C.D., McCulloch C.E., and Johnston S.C. NIH Disease Funding Levels and Burden of Disease. *PLoS One*. 2011; 6:e16837.

² Sampat B.N., Buterbaugh K., and Perl M. New evidence on the allocation of NIH funds across diseases. *Milbank Quarterly*. 2013 Mar;91(1):163-85

also devoted to developing scientific resources and infrastructure as well as NIH's commitment to training the biomedical workforce.

A) How often do you and the NIH Institute and Center Directors jointly review scientific performance objectives toward NIH-wide scientific goals and objectives?

Answer:

The NIH leadership, including the IC Directors, reviews scientific progress on a continuing basis through meetings with staff, workshops, and reviews by IC Advisory Councils and Boards. Information from such reviews is shared and discussed during the annual Leadership Forum, biweekly IC Directors' meetings, and biweekly steering committee meetings, as well as during meetings of the Advisory Committee to the Director and the Council of Councils. Scientific progress is also assessed as part of the development of updated or new strategic plans. In addition to this ongoing progress review process, the NIH assesses progress toward meeting scientific performance measures through the Government Performance and Results Act (GPRA) reporting that occurs as a part of the annual budget process. Information on a representative set of scientific research outcome measures is reported by all ICs to the NIH Office of the Director (OD), and in turn to HHS, OMB, and Congress with the Congressional Budget Justification.

B) Will NIH commit to publishing an NIH-wide scientific strategic plan, the funding decision criteria, and the measurable goals and objectives NIH leadership will use to help us and the public better understand NIH's funding allocation decisions?

Answer:

NIH appreciates the need for coordinated planning efforts to ensure that its scientific research investments are effective and synergistic. The forthcoming FY 2014 Congressional Appropriations Committee Report describes how the agency employs NIH-wide strategic planning processes, how scientific goals are measured and tracked, and how portfolio balance is achieved. Please see the response to Question A above, which provides details about NIH's funding criteria.

3) Administrative Burden Reduction Workgroup

Last spring I conducted a round table in Atlanta with a number of research and public health institutes in Georgia with Dr. Collins of NIH and Dr. Friedan of CDC.

I very much enjoyed the discussions and learning about the variety of on-going research.

It was interesting hearing feedback from the researchers. I was surprised by number of institutes that expressed a desire to have NIH take some steps to reduce the administrative burden that is placed on institutions that receive NIH supported grants and awards.

The 2014 Omnibus includes language requesting NIH establish an Administrative Burden Reduction Workgroup to develop a method to track and measure the administrative burden

5

on entities participating in NIH supported activities with the goal of developing a plan to reduce such administrative burden as practicable.

Have you had an opportunity to charter this working group yet?

How is NIH reaching out to universities, not-for-profits, and institutes to engage a cross section of the organizations that receive NIH funding into this workgroup?

Can you give me a sense of the types of burdens and if you expect reduction will result in requests for legislative relief or regulatory changes to lessen unnecessary administrative burden on these organizations?

Answer:

NIH is committed to implementing processes for administrative oversight that minimize the burden for grantees and is involved in a number of activities to accomplish this objective.

NIH will continue to participate fully in opportunities to partner with universities, not-for-profits, and research institutes that receive federal grant support to develop methods to track and measure the administrative burden on entities. The Federal Demonstration Partnership (FDP) is an association of federal agencies, academic research institutions with administrative, faculty and technical representation, and research policy organizations that work to streamline the administration of federally sponsored research. The FDP sponsored a Faculty Burden Survey in 2007 and again in 2012 to assess the nature and magnitude of administrative burden among its member organizations. NIH leadership will continue to extend invitations to the FDP to partners to identify ways to reduce burden using the results of their recent survey, and to plan future assessments for ongoing tracking and measurement of administrative burden.

The Research Business Models (RBM) Working Group is an interagency working group of the Subcommittee on Social, Behavioral and Economic Sciences (SBE) of the Committee on Science (CoS) a chartered committee of the National Science and Technology Council (NSTC). RBM Working Group objectives include streamlining business models for the conduct of scientific research sponsored by the Federal government. Among the accomplishments of the RBM working group has been the development of a standardized uniform format for reporting performance on Federally-funded research projects. Development of standard reporting categories will facilitate the development of a common electronic solution for collecting information in lieu of collecting it through numerous agency-unique reporting forms currently used. The new Research performance Progress Report has directly benefitted award recipients by making it easier for recipients to administer Federal grant programs through standardization of the types of research information required in performance reports. The RBM Working Group also sponsored a task force to develop recommendations for a unified set of costs principles for university-sponsored research that ultimately was included in the "Unified Guidance" which was issued by the Office of Management and Budget later in 2013.

The NIH Scientific Management Review Board (SMRB) has also been asked to consider this topic. The SMRB was established pursuant to Sec. 401(e) of the PHS Act to advise

HHS and NIH officials on the use of organizational authorities provided in the Act. At its meeting on May 7, 2014, the SMRB was charged with recommending ways to further optimize and streamline the process for reviewing, awarding, and managing NIH grants in a way that maximizes the time researchers can devote to research while still maintaining proper oversight. The SMRB will address the administrative requirements placed on recipient institutions and other parties involved in the grant-making process (i.e., scientific reviewers, Council members, and NIH staff). It is anticipated that the SMRB will complete its deliberations by spring 2015.

Modifications to the Common Rule (regulations for research involving human subjects shared by eighteen federal agencies) have been proposed by the Office of Science and Technology Policy and the Department of Health and Human Services. The Common Rule ANPRM was written to revise and update the system for regulating research involving human subjects to better protect human subjects who are involved in research, while facilitating valuable research and reducing burden, delay, and ambiguity for investigators. Some of the proposals intended to decrease administrative burden include: promoting the use of a single IRB of record for multi-site studies; not requiring continuing review for minimal risk research; and, promoting consistent policies among Common Rule signatory agencies. The ANPRM in its entirety is available at: <http://www.gpo.gov/fdsys/pkg/FR-2011-07-26/html/2011-18792.htm>. As the work of the SMRB progresses, other impacts of regulatory mandates on grantee administrative burden may be considered.

4. Replication of Scientific Research

Dr. Collins, I was pleased to see that NIH is starting to publically stress the importance of experimental rigor and transparency of reporting of research findings in order to enhance the ability of others to replicate them. To ensure trust and integrity of the public in efforts of the scientific enterprise requires rigor, reporting, and accountability.

In your January 2014 Nature article, you assert that poor training is the problem for the increased lack of scientific integrity – I would like to understand the data that support that assertion.

Plus, please highlight what is in the FY 2015 budget request to stress the importance of experimental rigor and transparency of reporting of research findings and how will success be measured?

Question:

Dr. Collins, I was pleased to see that NIH is starting to publically stress the importance of experimental rigor and transparency of reporting of research findings in order to enhance the ability of others to replicate them. To ensure trust and integrity of the public in efforts of the scientific enterprise requires rigor, reporting, and accountability.

In your January 2014 Nature article, you assert that poor training is the problem for the increased lack of scientific integrity – I would like to understand the data that support that assertion.

Answer:

Poor training of researchers in experimental design does contribute to issues with experimental rigor and the transparency of reporting of research findings. Multiple articles have highlighted the problems in how methodological approaches are potentially designed and reported.^{3,4,5} To aid in redressing the issue of poor or inadequate training, NIH is developing a training module on enhancing reproducibility with an emphasis on experimental design that will be piloted with NIH intramural postdoctoral fellows later this year, and provided to the extramural community online in its final form.

Flaws in how scientific research is conducted and communicated through publications can be partially, but not completely, attributed to poor training in experimental design. Other factors affecting reproducibility of research findings include increased emphasis on making “big picture” statements rather than presenting the fine details of the work; difficulty in publishing negative data or papers that point out scientific flaws in previously published work; potential perverse incentives for investigators to “publish or perish”, and publications that do not report basic elements of experimental design. In addition to the training module mentioned above, NIH efforts in this arena also include pilots at the Institute or Center (IC)-level to evaluate the scientific premise of grant applications, use checklists to ensure more systematic evaluation of grant applications, explore options to reduce perverse incentives, and support replication studies in the case of preclinical studies being considered for translation into clinical trials.

Problems with experimental rigor and the transparency of reporting of research findings are often associated with concerns of an increased lack of scientific integrity. Scientific integrity, in turn, brings up connotations of research misconduct, which is one of the potential causes of problems with reproducibility, but it comprises a small fraction. In 2013, the Office of Research Integrity (part of the U.S. Department of Health and Human Services) received approximately 450 allegations of research misconduct (not all of these allegations involved Public Health Service (PHS)-supported research or were within ORI’s definitional jurisdiction of falsification, fabrication, or plagiarism). ORI’s oversight review of 40 institutional investigations of potential research misconduct resulted in 12 PHS research misconduct findings. Thus, the issues concerning the rigor of research findings that are caused by this type of fraudulent behavior are serious, but outweighed by those issues caused by the complex array of factors listed above.

Question:

³ <http://www.sciencedirect.com/science/article/pii/S0166223607001804>

⁴ <http://www.ncbi.nlm.nih.gov/pubmed/22796459>

⁵ <http://www.ncbi.nlm.nih.gov/pubmed/17032985>

Plus, please highlight what is in the FY 2015 budget request to stress the importance of experimental rigor and transparency of reporting of research findings and how will success be measured?

Answer:

Experimental rigor and transparency are considered an integral component of what is expected and undertaken by the NIH ICs in pursuit of their IC-specific missions and the broader mission of the NIH. As a result, NIH efforts to address these issues are not specifically highlighted within the FY 2015 budget request.

With regard to how success will be measured, the efforts mentioned above will include evaluation by the IC or Office implementing the pilot. For example, in the case of the training module, which is an OD-led pilot, it will include pre-testing and post-testing of participants to evaluate the effects of the training, which will aid in optimizing the training in subsequent iterations. All of the information gained through the pilot activities will be used to decide which approaches could be implemented NIH-wide, which would be best kept at the IC-level, and which should be eliminated altogether.

5. Allocation of Funds toward Health Care:

Question:

Dr. Collins, in the NIH FY 2015 budget request (page ES-29 of the NIH Overview) it provides a breakout of the NIH funds spent relative to HHS Strategic Goals. It shows the funding for “Accelerate the process of scientific discovery” dropping by \$1 billion from FY 2014 to FY 2015 at \$26.8 billion.

Plus, it shows NIH is spent \$745 million in FY 2013 to “Strengthen Health Care.” In this category the NIH focus was in the area of “Emphasize primary & preventive care linked with community” and the spending is projected to increase to \$1.6 billion or by \$1 billion in FY 2015 over FY 2013.

It appears that these “Strengthen Health Care” spending is not bio-medical research or discovery activities as they listed in different categories.

Can you please explain:

- 1) I believe this category in the HHS strategic plan is where HHS focuses its Obamacare and health reform activity. Can you assure us that NIH is not funding any Health Reform or ObamaCare related activity?

Answer:

Yes. I can assure you that NIH has not funded, or has any plans to fund, activities directly related to implementation of uninsured coverage mandates, insurance exchanges, subsidies or other regulatory elements of the Patient Protection and Affordable Care Act (ACA) enacted in 2010.

The FY 2015 NIH Congressional Justification (CJ) exhibit entitled “Budget by HHS Strategic Goal” (page ES-29) meets a requirement to fully distribute the President’s Budget (PB) request across the HHS strategic goals. The increase shown for HHS Strategic Goal 1 (Strengthen Health Care) did not reflect any change in resource allocation, just a long-standing methodology that has become unreliable due to changing circumstances. There was no intent to reduce NIH’s organizational emphasis on Strategic Goal 2 (Advance Scientific Knowledge and Innovation) in FY 2015. Since FY 2006, the funding distribution in this exhibit has been developed by associating NIH’s Government Performance and Results Act (GPRA) performance measures with the HHS strategic goals, and extrapolating from the cost of those measures in order to prorate the entire NIH budget. The measures themselves are listed in the CJ, starting on page OA-40. NIH implemented a representative approach to GPRA reporting in response to OMB’s mandate that the agency revise its comprehensive goals to bring them to a lower level of aggregation and to add greater specificity of desired outcomes. This representative approach was implemented in FY 2003. Since the GPRA performance measures represent a very small portion of the NIH budget (see table below), minor changes in the number, cost or budget mechanism of measures can cause major shifts in the distribution of the overall NIH budget by strategic goal. The FY 2015 budget was a transition year in terms of the number of GPRA measures and cost (both of which decreased); this shift amplified the impact of changes to the measures on the budget distribution.

For example, a single GPRA measure has been associated with Goal 1 (Strengthen Health Care) for the past three years, and the cost of this measure declined from \$49.5 million in FY 2014 to \$46.3 million in the FY 2015 President’s Budget -- a decrease of nearly 6.5 percent. However, since the total extramural dollars for GPRA measures affiliated with other goals decreased by 41.5 percent in FY 2015, the dollars assigned to Goal 1 became a larger portion of the sample. When extrapolated to the entire NIH budget, this caused the estimate for Goal 1 to increase from \$1.003 billion to \$1.612 billion. As a result, the increase did not reflect any change in resource allocation, just a long-standing methodology that has become unreliable due to changing circumstances.

There was also an increase in the estimate for Goal 4 (Increase Efficiency, Transparency, and Accountability of HHS Programs) from \$1.011 billion to \$1.581 billion. Most of the change resulted from the completion of one Goal 2 (Advance Scientific Knowledge and Innovation) measure that had \$14.9 million attributed to the Research Management & Support (RMS) mechanism. Removing that measure from the sample increased the percentage of GPRA measures associated with Goal 4 among the measures with RMS funds, thus causing a shift from Goal 2 to Goal 4 of \$418 million in extrapolated RMS funds at the NIH level. There was no intent to estimate a reduction of NIH’s emphasis on Goal 2 in FY 2015.

For FY 2016, NIH intends to revisit the methodology for this budget exhibit in order to reflect the distribution of budget dollars by HHS Strategic Goal as accurately as possible.

| Fiscal Year | FY 2013 | FY 2014 | FY 2015 |
|---------------------------------|--------------------------------------|--------------------------------------|--------------------------------------|
| Number of Measures with funding | 30 (25 in Goal 2) | 25 (20 in Goal 2) | 24 (17 in Goal 2) |
| Cost of those Measures | \$1.65 billion | \$1.31 billion | \$0.89 billion |
| Total Budget | \$29.15 billion (17.7 multiplier) | \$30.15 billion (23.0 multiplier) | \$30.36 billion (34.1 multiplier) |

- 2) What line of research or activity is and will be funding in this category?

Answer:

As indicated above, the number of GPRA measures associated to a HHS Strategic Goal can vary, as can the number of ICs participating in projects supporting the goal, as well as the number of years the measure is considered relevant. At present, there is a single measure supporting Objective 1-C, which will be active through FY 2018. One lead IC (NIMH) and three contributing ICs – NIDA, NINR, NIAAA – are tracking the amount spent or to be invested to identify three effective system interventions generating the implementation, sustainability and ongoing improvement of research-tested interventions across health care systems. Funding for this activity and similar projects possess a definite research nexus as opposed to a healthcare delivery or regulatory dimension.

- 3) How does NIH and HHS coordinated multi-agency efforts to not duplicate what other agencies are already funded to work in this area perform?

Answer:

For the past several years, great progress has been made to ensure efficient coordination between agencies located within the Department of Health of Health and Human Services (especially with bureaus funding biomedical research, e.g., the Centers for Disease Control and Prevention (CDC).

Given the complementary missions of the CDC and the NIH, the two agencies often work closely together to build on each other's respective strengths and achieve shared objectives. While the NIH conducts and funds basic and applied biomedical and behavioral research, the CDC engages in health promotion, prevention of disease, injury, and disability, and preparedness for new health threats. The CDC and the NIH partner on a diverse range of activities, such as co-funding research initiatives, co-developing health surveys, co-sponsoring scientific meetings and workshops, and engaging in interagency planning activities conducted through numerous committees and working groups.

Examples of ongoing collaborative activities include:

- NIH, with the National Institute on Allergies and Infectious Disease (NIAID) as the lead, collaborates with the CDC and other Federal partners to address the important issue of antimicrobial resistance. NIAID co-chairs, along with the CDC and the FDA, the Federal Interagency Task Force on Antimicrobial Resistance (ITFAR). Recently, NIAID collaborated with CDC to conduct a clinical trial to identify new treatment options for gonorrhea, which is increasingly becoming antibiotic resistant.
- The CDC and the NIH maintain a strong and active presence within the Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) by participating in multiple teams and committees to ensure that the results of NIH-supported research can be translated rapidly into safe and effective medical countermeasures for emerging and reemerging infectious disease threats and to ensure coordination of scientific activity with PHEMCE partners. For example, NIAID collaborated with the CDC, FDA, and the Biomedical Advanced Research and Development Authority to rapidly develop and test a vaccine for 2009 H1N1 pandemic influenza, including in special populations such as the elderly and children.
- The National Health Interview Survey (NHIS) (coordinated by the National Center for Health Statistics (NCHS) within the CDC) has collected data on the nation's health since 1957 through personal household interviews. The NIH provides funding for the survey and several of the NIH Institutes sponsor special supplements or help design questions for the survey. NHIS results provide data to better track health status, health care access, and progress toward achieving national health objectives.
- In a similar collaborative effort, the NIH also supports the CDC's National Health and Nutrition Examination Survey (NHANES). NHANES is a large program of studies designed to assess the health and nutritional status of adults and children in the U.S.
- The NIH and the CDC also partner on surveillance efforts related to sudden death in the young (SDY) and recently launched a new registry to track cases of SDY (up to age 24), including sudden cardiac death and sudden unexpected death in epilepsy (SUDEP).
- The NIH, in collaboration with the CDC and the Indian Health Service, continues to support the Diabetes Prevention Program, started in 1994. This long-term outcomes study has shown that diet and exercise or the diabetes medication, metformin, can delay the onset of diabetes by 10 years.

- To advance and accelerate progress in addressing the nation's childhood obesity epidemic, the NIH and the CDC, along with the USDA and the Robert Wood Johnson Foundation, formed the National Collaborative on Childhood Obesity Research (NCCOR). Capitalizing on each other's strengths, the NCCOR partners aim to improve the efficiency, effectiveness, and application of childhood obesity research. Specifically, NCCOR seeks to:
 - increase surveillance of childhood obesity,
 - identify, design, and evaluate practical and sustainable interventions, and
 - support coordination and collaboration to halt and reverse childhood obesity.
- Sponsored by the CDC, HRSA, the NIH's Office of AIDS Research, NIAID, and National Library of Medicine, AIDSinfo is a Web resource that offers access to the latest, federally approved HIV/AIDS medical practice guidelines, HIV treatment and prevention clinical trials, and other research information for health care providers, researchers, people affected by HIV/AIDS, and the general public.
- Administered by the NIH, the Disaster Research Response (DR2) Project coordinates efforts of the CDC, the Agency for Toxic Substances and Disease Registry, the FDA, and the HHS Office of the Assistant Secretary for Preparedness and Response to create a disaster research system consisting of coordinated environmental health disaster research data collection tools and a network of trained research responders. This effort will provide invaluable lessons and platforms for advancing timely post-disaster research activities.
- Using a database created by NIH to publish sequences of 100,000 bacteria known to cause foodborne outbreaks, the NIH, CDC, and FDA are piloting a project to operate a pipeline for rapid pathogen identification of the bacteria involved in specific outbreaks and facilitate development of tests to aid diagnosis and treatment of affected people.
- The Dietary Supplement Label Database is a searchable database of information taken from the labels of more than 20,000 dietary supplement products. The NIH collaborates with the CDC, USDA, FDA, and DoD to update the database monthly, providing important health information to the public.

- NIAID is developing a protocol for a placebo controlled clinical trial to assess the effectiveness of the antifungal drug fluconazole to treat Valley Fever in the context of community acquired pneumonia, a serious and potentially life-threatening condition. NIH anticipates that the trial will begin in mid to late 2015. To help NIH with identifying patients for the trial, CDC has identified potential patient enrollment sites, including a group of clinical practices that treat community acquired pneumonia in the areas of California and Arizona in which Valley Fever is highly endemic.

4) What basic bio-medical research is being reduced to allow for this funding?

Answer:

There is no reduction in biomedical research funding since the NIH activities associated to HHS objective 1-C involve investigation of key factors influencing the scaling up of research-tested interventions across large networks of services systems such as primary care, specialty care and community practice. These types of efforts are consistent with the NIH mission to conduct or support research designed to understand the basic biology of human health and disease and then apply this understanding towards designing new approaches for preventing, diagnosing, and treating disease and disability.

6. Funding for cancer research not a higher priority?

Dr. Collins: The lifetime risk of cancer is 1 in 2 for men and 1 in 3 for women. These risks are even greater for people with certain risk factors. More than 1.6 million cancer cases are expected to be diagnosed in the U.S. this year, and about 585,000 Americans are expected to die from the disease. Annually, cancer is also estimated to cost the U.S. economy more than \$216 billion in direct treatment costs and lost productivity. With 77 percent of cancer diagnoses occur in people over age 55, incidence rates and the financial costs associated with cancer are expected to grow dramatically in the coming years.

Yet somehow cancer research funding as a share of the NIH budget has declined while the scientific and public health need has gone up. In the late 1990s, NCI's budget made up 18.7 percent of the NIH budget. Today, it is 16.4 percent of the NIH budget. That decline has reduced NCI's funding by \$680 million below what it would have received in FY 2014 if its share of NIH's total budget had been maintained.

Despite the limitations on its resources, we have seen great progress in cancer research during this time. Immunotherapies, treatments that harness the body's own immune system to fight the disease have become available for a select number of cancers. Genetic subtypes of cancers have been identified, which for some has led the ability to rule out ineffective treatments and identify the one that will be most effective.

There is a great deal of concern that the pace of progress is slowing, that we'll be unable to fully utilize the data generated by the Cancer Genome Atlas, or that progress will remain painfully elusive for the most challenging types of cancer.

Given the breadth of scientific challenges and opportunities, and that we face a population that is constantly growing and aging, how is funding for cancer research not a higher priority?

Answer:

The NCI has received a slightly lower portion of the overall NIH budget in recent years because some NIH funds have been assigned to trans-NIH efforts like the Brain Research, through Advancing Innovative Neurotechnologies (BRAIN) Initiative, and Alzheimer's disease research. Also, in the years immediately after the September 11, 2001 attacks on the U.S., a new research priority arose to address the needs to improve the availability of medical countermeasures against potential biological, nuclear, radiological, and chemical threats, which had a significant impact on the relative allocation of resources among the ICs. Furthermore, while NCI is responsible for more than 80 percent of the NIH cancer research investment, there are multiple ICs working on cancer research, either independently or in collaboration with the NCI.

7. Leveraging Basic Science and Technology to Transform Medicine

Question:

In previous years, NIH has stated how leaps in technology reduced the cost of sequencing a whole genome from \$100 million to approximately \$1,000 today – making the move toward more personalized medicine more affordable. Isn't this type of technological leap possible in other areas of medicine too, holding the potential to bend the cost curve in the clinical setting and building a technology-enabled health system based on basic science to prevent diseases?

Answer:

Numerous emerging technologies have the potential to bend the cost curve in the clinical and health care system. Point-of-care testing technologies have become invaluable tools in the diagnosis and management of a variety of diseases and conditions. Their ability to assess clinically relevant information in rapid fashion could transform the delivery of health care in ways that expand access to care, enhance therapeutic precision, and improve cost effectiveness. The use of digital information technology such as wireless technologies, "smart" phones with internet capability, and wearable wireless sensors has the ability to transform medical care by allowing clinical information to be collected instantaneously, also at the point of care. Validation of point-of-care devices against standards of care will promote greater utilization, and can contribute to personalizing medicine, improving care, reducing costs by helping medical practitioners to assess, predict and/or diagnose medical conditions readily and inexpensively.

One specific project that aims to make a technological leap and bend the cost curve is the development of integrated microphysiological systems comprised of human cells for testing drug efficacy and toxicity in human health and disease. These microdevice systems, also known as human tissues-on-a-chip, are also expected to further our understanding of disease etiology and lead to improvements in the quality and numbers of novel therapies,

resulting in better clinical care. The tools have the potential to reduce significantly the overall cost of drug development by making more accurate predictions of a compound's safety and efficacy in humans.

In the clinical setting, we expect significant economies of scale once electronic health records (EHRs) are fully integrated and more data including DNA sequence data, from lower cost sequencing technologies, can be routinely part of patient records and implemented into care in a real way. Advances in the storage and utilization of informatics coupled with rich data from EHRs and other data sources are expected to enable technological leaps in how basic science can be integrated into a personalized approach to medicine.

Question: How can NIH increase its focus on preventative medicine to leverage basic biomedical science and technology?

Answer:

Supporting research on strategies for prevention and preventive medicine is part of the NIH mission to protect and improve health. All of the health-related NIH Institutes and Centers balance their portfolios to include studies on prevention of human diseases and conditions and research to understand the basis for human health. In addition, NIH's Office of Disease Prevention (ODP) provides leadership for the development, coordination, and implementation of prevention research in collaboration with NIH Institutes and Centers and public and private partners. For example, ODP is responsible for the "Pathways to Prevention" program, which identifies research gaps in specific scientific areas, finds methodological and scientific weaknesses in those areas, and conducts unbiased, evidence-based assessments of complex public health issues – all focused on stimulating progress in those research areas.

Leveraging basic biomedical research and technology is essential to devising new strategies for preventive medicine and examples abound. The National Institute of Biomedical Imaging and Bioengineering (NIBIB) established the Quantum Grants Program to make a profound (quantum) impact on the prevention, diagnosis, or treatment of a major disease or national public health problem through the development and implementation of biomedical technologies within 10 years. One of these NIBIB Quantum Grant supported projects seeks to greatly reduce cancer deaths due to metastases by identifying localized cancers before they metastasize. This may be achieved through the development of advanced microfluidic and molecular techniques for identifying, characterizing, and counting extremely small quantities of circulating tumor cells. The clinical use of circulating tumor cells as a sentinel for early detection of invasive cancer has long been recognized, but sufficient technology does not yet exist to pursue this promising diagnostic approach.

Basic and genetic studies of Alzheimer's disease and imaging research facilitated by the Alzheimer's Disease Neuroimaging Initiative are generating new insights into the biological underpinnings of this complex disorder and paving the way for the development of promising strategies aimed at preventing Alzheimer's. In other areas, high throughput

sequencing technologies and access to large data sets in NIH repositories such as dbGaP have facilitated researchers in finding gene variants that play a role in disease and developing diagnostics to advance disease risk assessment and other tools that may help prevent the development or worsening of disease. NIH research is also generating important insights into the prevention of diabetes. Studies funded through the Diabetes Prevention Program have shown that lifestyle changes, such as diet and physical activity, can lower the risk of developing type 2 diabetes by 58 percent in adults at high risk for diabetes.

Question: Are there changes to policies or procedures that can accelerate the coordination and harmonization of organizations like NIH, academia, and industry to transform basic science in the preventive digital medicine?

Answer:

Federal agencies, academia, and industry are highly motivated to coordinate and harmonize activities to transform the knowledge gained through basic science into preventive medicine in the digital age. NIH is supporting transdisciplinary research in partnership with other agencies and private industry on the collection and validation of digital-based images and data for health-related applications. Digital data from wearable devices, sensors and other sources have the potential to yield new insights into the factors that lead to disease or promote improved health through real-time actionable measures. As these nascent partnership activities grow and mature, NIH will, as needed, revise and augment policies and procedures that will further accelerate this area of collaboration.

8. Future of Medicine and the Role of NIH

Question: What does the next 100 years of medicine look like through the integration of technology and medicine? What role will this integration play in the improvement of patient care?

Answer:

Imagining the future of medicine and how it will be affected by technology over the course of a century is a challenging question to address. However, given advances of the past century, we can be fairly confident in predicting that science and technology will have a transformative effect on medicine during the 21st century.

Looking forward over the next decade, we expect technologies to become increasingly integrated into medicine and be a significant factor in the improvement of patient care. Healthcare systems will draw on a state-of-the-art information technology infrastructure to apply knowledge from basic research and electronic health records (EHRs) and contribute to the development of evidence-based practices. As genome sequencing technology continues to develop and the cost of generating and analyzing genomic information continues to decline, this information increasingly will be recorded in EHRs and used in clinical care and research, which will accelerate the advent of personalized medicine.

Further use of EHRs will drive the development of policies and regulations on patient privacy and advance the application of informed consent for ground breaking pragmatic clinical research.

Other promising emerging technologies may also play an important role in medicine in the future. We expect the continued development of point-of-care technologies will allow testing for many clinically relevant variables away from hospitals and other sites of clinical care.⁶ Rapid advances in neural interfaces will allow disabled individuals the ability to control their own bodies or internal or external devices and lead fuller and more productive lives.⁷ 3D printing technology, in conjunction with tissue engineering, is currently being evaluated for diverse uses including the production of prostheses and synthetic organs.⁸

Question: What is the appropriate role of NIH in supporting technology-focused science?

Answer:

NIH has a significant role in funding technology-focused science and in supporting and developing innovative programs that focus on advanced technologies. Technology-focused science has been funded through the NIH Common Fund Program, as well as the Small Business Innovation Research (SBIR) and the Small Business Technology Transfer (STTR) Programs, which stimulate innovation and facilitate translation of basic science innovations into commercialized products and technology. The establishment of public-private partnerships and coordinated efforts with other federal agencies will contribute to translating technology-focused science into the clinical setting.

Question: How can we streamline the “discovery” at other Institutes with the innovation from technology-focused Institutes, and then on to private sector partners?

Answer:

Several NIH initiatives have focused on streamlining discovery and engaging the private sector. For example, the Discovering New Therapeutic Uses for Existing Molecules program⁹ developed template documents for establishing agreements between universities and industry partners.¹⁰ The Accelerating Medicines Partnership is a collaboration between NIH, industry, and non-profit organizations to reduce the time and cost of developing new

⁶ See <http://www.nibib.nih.gov/research/featured-programs/point-care-technologies-research-network>

⁷ See <http://www.ninds.nih.gov/research/npp/>

⁸ See <http://www.nih.gov/researchmatters/may2013/05202013ears.htm>

⁹ See <http://www.ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/therapeutic-uses.html>

¹⁰ See <http://www.ncats.nih.gov/research/reengineering/rescue-repurpose/therapeutic-uses/agreements2014.html>

diagnostics and therapies.¹¹ The NIH Centers for Accelerated Innovations program, in partnership with the U.S. Patent and Trademark Office, was established to facilitate the progression of technology from the laboratory to the market.¹²

9. NIH Mission and Structure

Question:

1) What is the proper balance at NIH for Basic Science?

Answer:

Funding basic research is a vital part of the NIH mission and, in FY 2014, NIH is expected to allocate about 54 percent of its research budget for basic research, a figure that has remained steady for many years.¹³ NIH accounts for more than half of all federal funding for basic research,¹⁴ and is the largest funder of basic biomedical research in the world. As such, we are committed to maintaining this significant investment, which enables the basic discoveries that lead to a better understanding of human biology, diseases, and conditions.

2) How can we ensure that dollars allocated towards discovery and innovation at NIH is not replacing private sector R&D dollars?

Answer:

NIH aims to complement and not replace private sector funding of biomedical research. Evidence of this is the regular hand off of promising research to private sector companies and other research entities willing to fund clinical trials and address the regulatory requirements necessary to bring treatments and cures to the market. In 2013, the NIH Intramural Research Program alone executed 180 licenses, issued 71 new Cooperative Research and Development Agreements (CRADAs), and had a total of 376 active CRADAs.¹⁵ In addition, economic analyses indicate that NIH funding of clinical research does not replace private sector R&D spending but rather stimulates greater private sector spending.¹⁶

NIH funds research in areas and in stages of development that might not otherwise be funded. For example, NIH research investment in rare and neglected diseases tends to continue into later stages of R&D because there is little financial incentive for other sectors to invest. The long and often resource-intensive process of advancing basic discoveries to

¹¹ See <http://www.nih.gov/science/amp/index.htm>

¹² See http://www.nhlbi.nih.gov/about/dera/otac/caip/caip_index.html

¹³ For more information, see [http://officeofbudget.od.nih.gov/pdfs/FY15/Basic%20and%20Applied%20FY%202002%20-%20FY%202015%20\(Transmit\).pdf](http://officeofbudget.od.nih.gov/pdfs/FY15/Basic%20and%20Applied%20FY%202002%20-%20FY%202015%20(Transmit).pdf).

¹⁴ Ibid.

¹⁵ For more information, see <http://www.ott.nih.gov/erma/tt-metrics>.

¹⁶ Toole AA (2007). Does public scientific research complement private investment in research and development in the pharmaceutical industry? *Journal of Law and Economics*, 50: 81-104.

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human application and regulatory approval requires the contributions and cooperation of all sectors within the biomedical research enterprise.

- 3) How should we use new data like new quantitative metrics, such as patent output, for budget/planning purposes or the allocation/re-allocation of funds across NIH institutes in Congressional funding decisions?

Answer:

NIH is carefully considering the recently released report from the agency's Scientific Management Review Board on ways to assess value of biomedical research (see <http://smrb.od.nih.gov/documents/reports/VOBR-Report-122013.pdf>). Among other things, the report notes that most NIH administrative data systems were designed to manage contracts and grants throughout their lifecycle while other technologies to assess research output and outcomes are in their early stages. This is due in large part to differing views on what quantitative metrics are the most appropriate to use to accurately capture the value of NIH's basic, translational, and clinical science portfolios. Our ultimate goal is to improve health; however, applying biomedical knowledge in health practice often falls to NIH's many partners in community and health care settings. Therefore, quantitative metrics that measure health impact would reflect not just NIH performance, but performance of its partners in the health care sector. Ultimately, finding quantitative metrics for how specific research studies result in health improvement is quite challenging and could include patents, measures of technology usage, as well as changes in clinical guidelines, insurance coverage of new procedures, and many other potential indicators.

During the peer review process and annual reviews of research progress, NIH reviews the investigator's productivity, typically reported in the form of peer-reviewed publications, to track the scientific progress being made and the quality of research. The use of new, quantitative metrics (e.g., patents, changes in clinical guidelines, etc.) would be difficult to apply to budgeting or allocation decisions because these metrics typically are available well after the period of active grant support and their ability to adequately capture the ultimate value of research investments has not been demonstrated. As such, the metrics may have little relation to the new science being proposed in an upcoming budget cycle. In addition, it is very unclear how these metrics would be linked to the basic science portion of the NIH portfolio where progress is measured in terms of new knowledge. Despite the challenges in quantifying science outcomes and predicting results, NIH is continually exploring new data and methods to improve program administration and decision making.

10. Jobs and Competitiveness

Question:

- 1) Historically, NIH is a discovery organization. However, it has focused more in the past five years on innovation. If there is a clear role at NIH for innovating, please explain the difference between Innovation and Discovery:

Answer:

From its roots in 1887, the NIH has been a research organization, and has a long history of driving scientific discovery and innovation. **Discovery is the act of uncovering new knowledge,¹⁷ whereas innovation is development of a new idea, method, or device.¹⁸** The two often go hand-in-hand. For example, a recent basic research innovation in gene editing techniques called CRISPR is allowing scientists to target genes for deletion, addition, activation, or suppression with better precision than ever before, which in turn spurs discovery. Discovery and innovation can occur at any stage of research or development, from the most basic science to the most applied. As an organization whose mission is improving the public's health, it is our public service obligation to be not only a discovery organization but also an innovation organization, striving for both at the same time to achieve the main goal of benefiting the public health.

2) How does NIH balance these two priorities?

Answer:

Because discovery and innovation are not mutually exclusive, NIH fosters both in a complementary fashion, without detriment to the other. To bring benefits to the public health, both discovery and innovation are best nurtured through a balanced and diverse portfolio of investments covering a mix of basic to applied, disease-focused to population-focused, cellular and animal model to human subject research, among many other factors.

3) What does NIH use as output metrics in order to maximize innovation and discovery generated by NIH?

Answer:

Innovations and discoveries made with NIH funding advance the NIH mission to seek fundamental knowledge about the nature and behavior of living systems and apply that knowledge to enhance health, lengthen life, and reduce illness and disability. Thanks in large part to NIH-funded medical research, Americans today are living longer and healthier. Life expectancy in the United States has jumped from 47 years in 1900 to 78 years¹⁹ as reported in 2011²⁰, and disability in people over age 65 has dropped dramatically in the past three decades.

Many scientific findings advance science incrementally in both the public and private sectors. Some innovations are directly applicable to human health, such as diet and life style changes that decrease the incidence of disease. Others require commercial development of drugs, biologics or devices by the private sector to advance public health. NIH evaluates scientific progress toward meeting these goals at numerous levels. In basic research, NIH considers scientific publications, their citation by other scientists, and scientific awards. NIH also considers patents and commercial licenses to be evidence of progress toward innovation and discovery, along with other indicators of

¹⁷ "discovery." *Merriam-Webster.com*. 2014. <http://www.merriam-webster.com> (19 May 2014).

¹⁸ "innovation." *Merriam-Webster.com*. 2014. <http://www.merriam-webster.com> (19 May 2014).

¹⁹ BR Bloom. *Nature*, v. 402 (supp.) pp c63-c64 (1999).

²⁰ DL Hoyert and J Xu. *National Vital Statistics Reports* v. 61 (6). Centers for Disease Control, 2012.

commercialization and societal uptake, such as clinical trials, FDA approvals, new clinical practice guidelines and clinical recommendations.

- 4) How does the focus on innovation and translation ensure that there is enough seed corn of basic research for future generations to translate?

Answer:

The primary source of basic research funding in the United States remains the federal government, which obligated approximately \$29 billion for basic research in 2011.²¹ NIH accounted for more than half of those basic research obligations.²² Funding basic research is a vital part of the NIH mission, and in FY 2014 NIH is expected to allocate 53.9 percent of its research budget for basic research, a figure that has remained steady for many years.²³ NIH is committed to maintaining this significant investment, which enables the basic discoveries and innovations that lead to a better understanding of human biology, diseases, and conditions.

However, the analogy between basic science and seed corn too cleanly separates the impacts of basic from applied research. New innovations and discoveries from basic science may constitute inventions that have commercial value. The discovery of new genes associated with a medical condition can be used in innovative commercial diagnostics, or to drugs to interrupt the action of the genes. NIH funding of Cohen and Boyer studies of how bacteria mate led to the unexpected invention/discovery of restriction enzymes. The patent was widely licensed to all companies using recombinant DNA to make biologic therapeutics. Other inventions more predictably arise from applied research into new vaccines and new cancer therapies.

NIH balances its research portfolio by ensuring a diverse range of activities covering basic, clinical, translation and technology development.

Research Project Grants: Research project grants (RPGs) are the primary mechanism for funding of investigator-initiated biomedical research. The use of RPGs as a mechanism of support covers the entire medical research continuum, from basic scientific research at the molecular and cellular levels to studies of human beings in both healthy and diseased states.

Research Centers: Research center grants are awarded to institutions on behalf of a program director and a group of collaborating investigators. The Research Centers program aims to integrate basic research with applied research and transfer activities; to promote research in the areas of clinical applications with an emphasis on intervention, including prototype development and refinement of products, techniques, processes, methods, and

²¹ National Science Board. 2014. *Science and Engineering Indicators 2014*. Arlington VA: National Science Foundation (NSB 14-01).

²² Table 4-14, National Science Board. 2014. *Science and Engineering Indicators 2014*. Arlington VA: National Science Foundation (NSB 14-01).

²³ For more information, see

[http://officeofbudget.od.nih.gov/pdfs/FY15/Basic%20and%20Applied%20FY%202002%20-%20FY%202015%20\(Transmit\).pdf](http://officeofbudget.od.nih.gov/pdfs/FY15/Basic%20and%20Applied%20FY%202002%20-%20FY%202015%20(Transmit).pdf)

practices; to develop and maintain the biotechnology and research model resources needed by NIH-supported biomedical investigators for conducting research; and, to assist minority institutions in improving their research infrastructure.

Intramural Research: Through the Intramural Research Program (IRP), NIH conducts basic and clinical research at its on-campus research facilities in Bethesda, Maryland, and at off-campus locations. Fundamental research performed by intramural scientists provides the basis upon which advances in medical and dental care are built. The "bench-to-bedside" approach adopted by the NIH Clinical Center in 1953 locates patient care units in close proximity to cutting-edge laboratories conducting related research, which facilitates interaction and collaboration among clinicians and researchers. Most importantly, patients and their families at the Clinical Center benefit from the signature elements of NIH (i.e. cutting-edge technologies, research programs, and compassionate care).

- 5) Is the number of patents a reasonable output measure for the innovation component of NIH spending?

Answer:

Measuring innovation is a complex problem. The number of patents resulting from NIH-funded research is only one of many potential indicators of innovation. Relying on number of patents alone to measure innovation would be an incomplete and potentially misleading picture of innovation from NIH spending.

- 6) Do you know what the link is between patent production and job production domestically?

Answer:

Most research on the relationship between patents and jobs links employment outcomes to the commercialized products resulting from patents.²⁴ The US Patent and Trademark Office reported that the 26 patent-intensive industries accounted for 3.9 million jobs in 2010 and that an additional job is supported elsewhere in the economy for every job in intellectual property-intensive industries.²⁵

http://www.uspto.gov/ip/officechiefecon/report_ip_and_the_us_economy.jsp

- 7) How about NIH supported patent production and job production?

Answer:

Commercialized products are typically attributable to multiple patents; patents, in turn, may be attributable to multiple funding sources. Thus, it can be difficult to quantify the relative contributions of each source of support, and since many of the patentable discoveries

²⁴ J. Rothwell, J. Lobo, D. Strumsky, M. Muro Patenting Prosperity: Invention and Economic Performance in the United States and its Metropolitan Areas. Brookings Policy Program, 2013.

²⁵ Intellectual Property and the U.S. Economy: Industries in Focus. US Department of Commerce, March 2012

resulting from NIH's basic research portfolio occur far upstream from the commercialized products, NIH's contribution may be underestimated.

NIH's basic research portfolio also provides direct support for jobs through salaries and stipends for the NIH research workforce. Thus, NIH research programs directly support employment and training opportunities, those employed with NIH funds make discoveries and innovations, which in turn provide the vehicles for commercialization and stimulate many more private sector jobs downstream from the initial NIH investment.

- 8) What private sector strategies would be applicable to NIH's innovation goals, while maintaining its core function to be a discovery agent?

Answer:

The roles of federal and private sector investments in biomedical research and development are complementary. In most cases, the appropriate role for federal agencies is to make research investments to benefit the public that may be too large or remotely isolated from an assured commercial return for the private sector to absorb. In the case of developing new therapeutic products and disease interventions that are both effective and commercially-viable, NIH must often interface with the private sector to ensure the efficient hand-off of scientific knowledge to its ultimate application in health practice. Rather than applying private sector strategies in the public sphere, NIH strives to bridge gaps in the therapeutic development pipeline and incentivize private sector investment in areas of public health need.

There are many intermediate steps between obtaining a patent and launching a product, particularly for FDA approved drugs, devices and therapeutics. Most inventions made with NIH-funded research are very early stage, and thus high risk investments. New biomedical products are rarely developed by the private sector without patent protection. A company must license the invention, obtain investment funding, engage in preclinical and then clinical trials to obtain FDA approval to market the product. From start to finish, this developmental pipeline can take well over a decade and has a failure rate of more than 95 percent, with many failures occurring late in the process.

NIH supports programs that aspire to accelerate much-needed therapeutic development and enhance the hand-off from the laboratory to the market, often through formal collaborations with industry. One such example is the Accelerating Medicines Partnership (AMP), which brings together the NIH, the Foundation for NIH, FDA, ten pharmaceutical companies, and a number of non-profit organizations. By optimizing how disease targets are identified and validated for drug design, the goal of AMP is to complement and enhance the private sector's ability to develop new diagnostics and therapies for patients.

Similarly, the goal of NIH's Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) Programs is to engage the small business community in efforts to bring biomedical knowledge and technology to the market. NIH's SBIR/STTR program has been successful at achieving this goal. The National Academies

of Science²⁶ have evaluated the SBIR program at NIH (and other agencies) and found that the private sector views SBIR-funded technologies as substantially lower risk than other innovation research ventures because these projects are subjected to a rigorous review process based on both scientific merit and commercial potential.

While the agency's primary mission is to improve health, NIH recognizes that commercialization of therapeutics originating in the academic laboratory is also an important economic driver for the nation. One study documented 153 FDA approved drugs and biologics developed by companies from inventions licensed by U.S. universities, research hospitals and the NIH intramural program combined.²⁷ Products based on licenses of inventions from the NIH intramural patenting and licensing program alone generated \$7 billion in sales to the licensee companies in 2013.²⁸ Another economic study based on licensing income from U.S. universities, hospitals and research institutes over 15 years estimated the contribution to GDP to be from \$86 billion to \$338 billion in 2005 US Dollars.²⁹ Through NIH's various efforts to hasten the movement of scientific knowledge from the laboratory to the market, the agency strives to improve public health as well as contribute to the economic engine of biomedicine.

11. NIH Preventative Research:

Question:

- 1) Dr. Collins, could you discuss the newly released NIH Strategic Plan for Prevention Research released last month? How will this change the way that NIH allocates its extramural and intramural funding and the balance with basic biomedical research?

Answer:

In February 2014, the NIH Office of Disease Prevention (ODP) released its first strategic plan which outlines the priorities that the Office will focus on over the next 5 years. The goal of this effort is to increase the scope, quality, dissemination, and impact of prevention research supported by the NIH. The ODP will achieve this goal by providing leadership for the development, coordination, and implementation of prevention research in collaboration with the NIH Institutes and Centers and with other partners. While the priorities and objectives outlined in the plan are designed to benefit the broader NIH prevention research community, the plan itself was developed as a tool for the ODP and does not represent a trans-NIH plan for prevention research.

²⁶ Charles W Wessner (Ed.) An Assessment of the Small Business Innovation Research Program at the National Institutes of Health National Research Council (US) Committee for Capitalizing on Science, Technology, and Innovation: An Assessment of the Small Business Innovation Research Program. Washington (DC): National Academies Press (US); 2009.

²⁷ A.J. Stevens, J.J. Jensen, K. Wyller, P.C. Kilgore, S.K. Chatterjee, M.L. Rohrbaugh. The Role of Public-Sector Research in the Discovery of Drugs and Vaccines. *N Engl J Med* 2011; 364:535-541.

²⁸ S.K. Chatterjee, M.L. Rohrbaugh; *Nat Biotechnol* 32:52-58, 2014

²⁹ Biotechnology Industry Organization. 2012. The Economic Contribution of University/Nonprofit Inventions in the United States: 1996-2010.

The ODP strategic plan will allow the Office to expand its influence by, for example, providing training in prevention methodology and developing new strategies for identifying research needs and opportunities – activities that may not otherwise be addressed by a single NIH Institute or Center but are important for advancing disease prevention research more broadly. Interest in disease prevention has grown, and the NIH has a responsibility to ensure that the best prevention science is supported to inform clinical and public health initiatives at the individual, organizational, community, and policy levels. The strategic priorities included in the plan will allow the ODP to play an important role in that process while giving NIH Institutes and Centers the flexibility to support prevention research within its extramural and intramural programs that best reflects its mission and state of the science of its programs. As the ODP implements its strategic plan and begins to examine specific areas within the NIH prevention portfolio, the NIH Institutes and Centers will be able to incorporate available information about needs and opportunities into its prioritization and decision making.

- 2) How much of the FY 2015 requests and in which Institutes and Centers is allocated towards the Prevention Research Strategic Plan implemented?

Answer:

The ODP is part of the Division of Program Coordination, Planning, and Strategic Initiatives (DPCPSI), which is located in the NIH Office of the Director. Funding for implementation of the Office of Disease Prevention Strategic Plan will be coordinated through DPCPSI and will be determined as part of the Division's program planning and priority setting process. Implementation of the ODP Strategic Plan will not affect allocation of funds for the NIH Institutes and Centers.

- 3) The new Plan calls for more systematic monitoring of NIH investments in prevention research. What is the base line measure for this activity currently, how will it be monitored, and how often to gauge progress?

Answer:

Currently, NIH prevention research is identified based on a broad definition of prevention that does not provide sufficient detail to examine progress by classifying studies into categories of interest. The ODP strategic plan outlines new processes for characterizing the NIH prevention research portfolio. For example, improved methods are needed that identify characteristics of studies – such as topic area, study design, population studied – and summarize the findings in a meaningful way for program planning and reporting. This more specific characterization of the prevention research portfolio, along with improved portfolio analysis tools, would enable identification of patterns and trends, as well as research areas that may benefit from targeted efforts by the NIH Institutes and Centers. Such categorization also would enable assessment of the progress and changes in prevention research over time. The new tools that ODP is developing will allow the Office to monitor investments and identify emerging areas of opportunity on an ongoing basis.

12. Heart Disease Research

I understand that by 2030 this number of people with heart disease is projected to increase to 8 million people. Please tell the Committee what NIH is doing to develop interventions that can prevent heart failure.

Answer:

Heart failure (HF) is a common condition associated with many hospitalizations, significant morbidity, 1 year mortality rates as high as 40%, and staggering effects on health care expenditures. It is estimated that by 2030 more than 8 million Americans will have HF at a cost of \$53 billion.

The NHLBI has a proud legacy of research that has revolutionized HF treatment. Ground-breaking studies such as SOLVD, which demonstrated that a class of medications called ACE inhibitors, not only reduced the occurrence of HF but also arrested its progression.

Despite significant advances in HF research, evidence indicates there is still a tremendous amount of work to be done to prevent this disease. In 2011 the NHLBI convened experts in the field who recommended a continued focus on studies to: (1) evaluate novel biomarker, imaging, and genetic markers to identify people at high risk for developing HF; (2) test novel interventions and/or ways to activate or enhance innate protective mechanisms to prevent the progression of HF; and (3) elucidate the epidemiology of HF, especially in women and minorities, who are more likely to have co-morbid conditions. The NHLBI-funded *CARDIA* study highlighted the importance of better understanding HF in minorities, demonstrating that when HF develops in young adults, it occurs at a rate 20 times greater in African Americans than whites, with hypertension and obesity as the leading risk factors.

NHLBI is making progress toward addressing these recommendations. For example, the expert panel's recommendation to develop HF biomarkers is the focus of an ongoing study called GUIDE-IT. This study is designed to test whether biomarker-guided HF medical management will improve outcomes compared to standard medical management.

Particularly exciting are new discoveries into the capacity of the heart to repair itself. A large inter-institutional team of NHLBI-funded investigators is building upon the previous work of Dr. Shinya Yamanaka, the 2012 Nobel Laureate, who discovered how to transform ordinary adult skin cells into cells that can then develop into virtually any other type of cell in the body. The research team is studying how these adult stem cells might contribute to repairing the heart and how these stem cells might also be used to reveal potential drug targets earlier in the pathway of disease that could possibly pre-empt or even prevent HF.

Adult stem cells are also being used by Dr. Joseph Wu and his team at Stanford to create heart cells and heart disease models that are being used as platforms to screen novel drugs (i.e., high throughput screening) to determine toxicities and potential for use as treatments, thereby expanding the search for drug therapies and doing so with more time efficiency. (*JAMA*) This new technology means that each one of us could potentially have our own stem cell line that could be used to identify the best treatment for us.

NHLBI is committed to supporting research focused on the prevention of HF with continued investments directed towards fundamental and clinical research aimed ultimately at eradicating this national epidemic.

13. Minority Participation in Cancer Clinical Trials

Drs Collins and Varmus, as you know, cancer is a major problem in Georgia, especially among African-Americans, who have disproportionately high rates of this disease. At the same time, African-Americans have very low participation rates in cancer clinical trials, accounting for only 5 percent of the total trial population. What are the implications for safety and efficacy of new cancer therapies in African-American patients given their extremely low participation in clinical trials?

What steps is NIH and NCI taking to ensure more African-American cancer patients enroll in clinical trials, particularly studies of novel immune therapies and molecularly targeted therapeutics?

Should NIH and NCI consider concentrating funding for clinical trials in regions of the country with large African-American populations?

Answer:

NIH recognizes the need for further research to reduce disparities and improve outcomes for underserved populations across both the disease spectrum and the continuum of care. The clinical benefits of NIH research highlight the importance of clinical trial participation for all patients. Recent literature suggests that racial and ethnic minorities are just as willing to be involved in research studies as the general population. Therefore, NIH strives to identify barriers that prevent underserved populations from enrolling in clinical trials and to create opportunities for them to participate in trials. Both the National Institute of Minority Health and Health Disparities (NIMHD) and the National Cancer Institute (NCI) have a number of efforts underway to support these goals.

NIMHD supports the project, Enhancing Minority Participation in Clinical Trials (EMPaCT) with the overall objective to increase recruitment and retention of racial/ethnic minorities into cancer therapeutic clinical trials. Using the well-established EMPaCT consortium and partnership with the American Cancer Society, the ultimate goal is the reduction of cancer-related health disparities. The two strategies employed to achieve this objective are: 1) enhanced education and training through web-based modules customized for investigators, research staff, referring physicians, and patient navigators and 2) implementation of a patient navigation program at each EMPaCT Center to facilitate enrollment and retention of minority patients into cancer clinical trials.

NCI has been working for nearly two decades to improve representation of minority patients in clinical trials through oversight of its Cancer Centers, and by development of the Community Clinical Oncology Program (CCOP) and Minority-Based CCOP network. The MB-CCOP program has consistently maintained high levels of minority accrual to NCI-supported clinical trials. A 2005 evaluation of the program showed that between 1995 and

2003, minorities comprised 51% to 67% of patients enrolled by the MB-CCOPs to treatment trials within NCI's national network, compared with less than 23% of the patients accrued by other members and affiliates. Many MB-CCOP sites serve communities with large African American populations, including Georgia Regents University in Augusta, the Tulane MB-CCOP in New Orleans, Louisiana, and the Virginia Commonwealth University MB-CCOP in Richmond.

Building on the success of the CCOP and MB-CCOP programs, NCI is currently implementing the NCI Community Oncology Research Program (NCORP), which supports cancer research in the community setting, with access to larger and more diverse patient populations. The goals of the new program include increasing enrollment of minorities into clinical trials in all areas, and focusing on disparities questions in clinical trials. NCORP will award grants to research centers, community sites, and minority/underserved sites, and each minority/underserved site will be required to accrue 30 percent of cancer patients from racial/ethnic minority or other underserved populations – like the MB-CCOP program, this will include sites that serve large African American populations.

Increasing accrual of populations underrepresented in clinical trials is also among the evaluation criteria for the community sites. NCORP will work in collaboration with the National Clinical Trials Network and the NCI-designated Cancer Centers to integrate disparities research questions across all study types, facilitate the participation of minority and underserved populations in clinical research, and accelerate knowledge transfer into clinical practice.

NCI's Center for Cancer Health Disparities has also supported research efforts to evaluate patient navigation, such as The Cancer Disparities Research Partnership (CDRP) program, which specifically tested the use of patient navigation for recruiting racially and ethnically diverse patients to clinical studies. CDRP navigation led to the accrual of a large number of patients into cancer clinical trials with representation from American Indians, African Americans, and Hispanics/Latinos.

14. Federal Asthma Guidelines

Question:

Dr. Gibbons,

- 1) Does NHLBI have any sense of timing on when a decision will be made regarding revising the current federal asthma guidelines?

Answer: NHLBI anticipates that a decision about whether or not to revise the current federal asthma guidelines will be made by the end of CY2014.

- 2) --Will the guidelines update be on the agenda for the June NHLBI Advisory Committee meeting?

Answer: Yes, at the June NHLBI Advisory Council meeting, the NHLBI Advisory Committee's Asthma Expert Working Group will present its report of the efforts to date to inform a decision about whether an update to the asthma guidelines is warranted.

3) --Will the comments that were submitted be made public? If so, when?

Answer: The NHLBI Advisory Committee's Asthma Expert Working Group has reviewed the comments received from the Request for Information as well as input from scientific experts and members of the National Asthma Education and Prevention Program (NAEPP) coordinating committee. The Working Group's report will be presented during the open session of the NHLBI Advisory Council meeting in June. The written report, which summarizes the comments that were submitted, will be available to the public at the meeting and will be posted on the NAEPP website after the meeting.

15. Rehabilitation Research

We understand NIH had a Blue Ribbon Panel on Medical Rehabilitation Research ("BRP") that made recommendations for related to the National Center for Medical Rehabilitation Research ("NCMRR") and rehabilitation science at NIH. Given the prevalence of people with disability and the aging population, Please provide a table that show the amount of funds spend by IC and at the NCMRR on rehabilitation research. Further, please explain how the Division of Program Coordination, Planning and Strategic Initiatives (DPCPSI) is used to coordinate with NCMRR to coordinate the NIH-wide rehabilitation research activity.

Answer:

As tasked, in December 2012 the Blue Ribbon Panel on Medical Rehabilitation Research (BRP) made its recommendations to the Director, NICHD, related to medical rehabilitation research supported by NIH. In response, in FY 2014 NICHD created a dedicated budget for the National Center for Medical Rehabilitation Research (NCMRR), which will remain under NICHD's purview. A working group representing several NIH Institutes and Centers (ICs) are further developing a plan for NIH funding of rehabilitation research, including increased coordination of research funding among ICs.

A table showing NIH FY 2013 expenditures in the overarching area of "rehabilitation" research is below; this information is publicly available on the NIH web site: http://report.nih.gov/categorical_spending.aspx. Please note, however, that the definition NIH uses for the purpose of reporting on rehabilitation research is broad, and includes research grants that do not fall within NCMRR's authority (e.g., rehabilitation for drug addiction). For FY 2014, a narrower definition for "medical rehabilitation" is being developed so that expenditures for this area can be more accurately reported.

| (Dollars in Millions) | FY 2010 | FY 2011 | FY 2012 | FY 2013 |
|-----------------------|---------|---------|---------|---------|
| Rehabilitation..... | \$458 | \$459 | \$449 | \$446 |

In addition, and in response to the BRP's recommendation to increase coordination, NICHD recently hosted the reinvigorated and expanded Trans-NIH Rehabilitation Research Coordinating Committee. Among other responsibilities, this group of NIH leaders and scientific experts is charged with identifying important research opportunities and collaborations. The discussion will provide the basis for a formal NCMRR strategic planning process, which will be further developed when the new NCMRR Director is selected later in 2014.

An essential part of DPCPSI's mission is identifying emerging scientific opportunities, rising public health challenges, or scientific knowledge gaps that merit further research. DPCPSI carries out this mission and coordinates research across a wide range of areas through six program offices with specific foci: Office of AIDS Research, Office of Behavioral and Social Sciences Research, Office of Disease Prevention, Office of Research on Women's Health, Office of Research Infrastructure Programs, and the Office of Strategic Coordination which manages the NIH Common Fund. While the division coordinates trans-NIH research in the programmatic areas within the purview of the program offices, DPCPSI does not coordinate all NIH-wide research, including medical rehabilitation research.

16. National Pediatric Research Network Act

Please provide an update of the activity started or planned since the enactment of the National Pediatric Research Act for each IC, which relates the activity covered by such act. Plus, what are the barriers toward implementing a pediatric research network?

Answer:

The National Pediatric Research Network Act amends the Public Health Service Act to allow the Director of the National Institutes of Health (NIH), in consultation with the Director of the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), to provide for the establishment of a pediatric research network. If such a network is established, the Director may utilize existing networks or centers already supported by NIH. If established, the law requires that an appropriate number of awards are made to those institutions conducting research on rare pediatric diseases.

To ensure that decisions are made with as complete information as possible, NICHD is in the process of finalizing a portfolio analysis of the pediatric research networks and centers programs supported by NIH. Most of the NIH Institutes and Centers support a variety of pediatric research projects within their areas of expertise, many of which include research networks or centers. A conservative estimate is that NIH currently supports approximately 100 pediatric research networks and centers programs. Additional analysis is ongoing to determine what pediatric diseases and conditions are covered, and which networks and centers include rare pediatric diseases and conditions. Moreover, NICHD will engage its ongoing program evaluation and assessment efforts to identify ways to address current obstacles and emerging opportunities in pediatric research.

In addition, NICHD officials have met with a wide range of patient organizations and professional societies to discuss the law, its implications for pediatric research efforts, and to gauge what obstacles the law might be intended to address. NICHD scientific and policy staff also have begun discussions about how to target these obstacles during the regular recompetition of currently funded pediatric research networks and centers programs through improved communication and data sharing across programs.

17. Improving Efficiencies

The budget proposal for NIH is relatively modest given the administration's purported commitment to innovation and research. It is critical to improve NIH's efficiencies. We look forward to the results of NIH's strategic planning activity. Please provide your suggestions on what NIH could do or steps Congress could take to improve how NIH can focus its limited resources on diseases prevention and treatment? What action should the Committee take to ensure that NIH ICs consolidate all of its training and mentoring programs in to a more centrally managed program? What changes can be made to policy or via legislation to make NIH supported clinical trials more efficient, cost-effective, and the results likely to be picked up by private industry without the private sector having to re-accomplish any of the trials or data to get FDA approval?

The budget proposal for NIH is relatively modest given the administration's purported commitment to innovation and research. It is critical to improve NIH's efficiencies. We look forward to the results of NIH's strategic planning activity.

Question:

- 1) Please provide your suggestions on what NIH could do or steps Congress could take to improve how NIH can focus its limited resources on diseases prevention and treatment?

Answer:

Achieving NIH's mission to improve public health through biomedical research requires a balanced and diverse portfolio of research investments, including research into promising preventive strategies as well as developing new and effective therapeutics. In addition to portfolio balance, the ICs are also responsive to public health needs, scientific opportunities, and budgetary considerations. The ICs continually engage in priority setting and strategic planning processes, including gathering stakeholder input, engaging independent scientific experts for advice, and conducting portfolio analyses, to better understand their portfolios, to assess research and health needs, and to identify emerging areas of scientific opportunity as well as existing knowledge gaps that merit increased funding. These processes together help ensure that NIH uses its resources effectively.

Question:

- 2) What action should the Committee take to ensure that NIH ICs consolidate all of its training and mentoring programs in to a more centrally managed program?

Answer:

Administrative oversight and policy development for NIH training programs is centralized within the Office of the Director as a way to ensure consistency across the entire program. Then, each IC manages its training programs considering IC specific programmatic priorities and objectives.

Question:

- 3) What changes can be made to policy or via legislation to make NIH supported clinical trials more efficient, cost-effective, and the results likely to be picked by private industry without the private sector having to re-accomplish any of the trials or data to get FDA approval?

Answer:

Improving the quality, efficiency, and cost-effectiveness of NIH-funded clinical trials is the subject of multiple current activities at NIH. For example, NIH is participating in efforts to modernize HHS regulations governing research on human subjects. One of the proposals under consideration would enhance clinical trial efficiencies by requiring the use of a single IRB of record for multi-site studies.

Increasing access to clinical trial data will also improve quality, efficiency, and cost effectiveness. Data on the outcomes of clinical trials can reduce inadvertent and unnecessary duplication of clinical studies and allow analysis of the results of multiple clinical trials of the same or similar interventions, thus providing scientists with more information regarding the potential benefits and harms of different interventions, information that can improve the design of future studies. Currently, many public and industry funded clinical trials are subject to Title VIII of the Food and Drug Administration Amendments Act (FDAAA) of 2007, a law that requires applicable clinical trials of drugs, biological products, and devices to register and submit summary results information to ClinicalTrials.gov, a publicly accessible database managed by the NIH National Library of Medicine. As required by the law, NIH will be issuing a proposed rule to expand the types of applicable clinical trials that must submit results, further define the data elements that must be included in summary result submissions and facilitate access to more data. ClinicalTrials.gov currently holds summary results data for more than 11,000 trials of drugs and devices and is the only public source of results data for many trials. To capture information about clinical trials that are not subject to the law, NIH intends to issue a policy that will call for all NIH-funded clinical trials to register and submit results to ClinicalTrials.gov. Facilitating access to all NIH-funded clinical trials will contribute to science and, ultimately, to the public health.

In addition to efforts to enhance the efficiency and cost-effectiveness of NIH clinical trials through policy initiatives, NIH's National Center for Advancing Translational Science (NCATS) is working in a number of areas to expedite the development of diagnostic, therapeutic, and prevention strategies through more efficient technologies, methods, resources and operational paradigms that catalyze clinical research progress. For example, the Therapeutics for Rare and Neglected

Diseases (TRND) program advances potential treatments for rare and neglected diseases to first-in-human trials, an approach known as “de-risking.” This strategy makes new drugs more commercially attractive to biopharmaceutical companies. NIH will continue to work toward enhancing the efficiency and cost-effectiveness of clinical trials through policy and programmatic initiatives.

18. Clinical and Translational Science Awards (CTSA)

Question:

In the fiscal year 2014 bill’s conference agreement, we worked on a bipartisan basis to support additional funding for the Clinical and Translational Science Awards (CTSA) program, and to request that NIH and NCATS focus carefully on implementation of the recommendations made by the Institute of Medicine (IOM) related to the CTSA.

1. Please provide an update on the implementation of IOM’s recommendations?

Answer:

In June 2013, the Institute of Medicine (IOM) issued a report following a review of the CTSA program. The report recommended that NCATS take a more active role in the program’s governance and direction, formalize the evaluation processes of the program, advance innovation in education and training programs, and ensure community engagement in all phases of research.

NCATS leadership is committed to implementing the recommendations of the IOM report. As a first step, NCATS has increased the programmatic and fiscal management of the grants that support this program and streamlined the way the consortium is governed, consulting closely with the CTSA PIs. For example, we have appointed a new steering committee that includes 12 CTSA PIs with staggered terms to replace the previous 90-member group.

In parallel, NCATS assembled a Working Group of its Advisory Council to provide advice on measurable objectives for the CTSA program. The group was tasked with developing clear, measurable goals and objectives for the program that address critical issues across the full spectrum of clinical and translational research (i.e., “what does success look like?”). The Working Group presented its report (<http://www.ncats.nih.gov/files/CTSA-IOM-WG-Draft-Report.pdf>) at the NCATS Advisory Council meeting in May. Its report addressed four of the seven recommendations in the IOM report and focused on: 1) translational workforce development, 2) engagement and collaboration with patients and communities, 3) integration of translational science across its multiple phases and disciplines within complex populations and across the individual lifespan, and 4) systemic improvements in methods and processes of translation. The measurable goals and outcomes in this report are serving as a guide for NCATS as it moves forward in developing and implementing strategies to strengthen the CTSA program and for measuring progress.

NCATS recently announced the selection of Petra Kaufmann, M.D., M.Sc. to head the NCATS Division of Clinical Innovation which includes the CTSA program. Dr. Kaufmann currently serves as director of the Office of Clinical Research at NIH’s National

Institute of Neurological Disorders and Stroke (NINDS) and brings a wealth of expertise across the translational sciences spectrum.

With the appointment of a permanent Director for the program, the recommendations of the IOM report, and the results of the Advisory Council working group deliberations, NCATS is poised to work closely with the CTSA community to improve the effectiveness and efficiency of the process of translation from scientific discovery through clinical research to improved health outcomes.

2. Also, please explain why the budget proposal recommends a cut to the CTSA program for fiscal year 2015 while the budget calls for increases for NIH and NCATS overall. This seems counter-intuitive given IOM's positive review and the identified need to continue to grow the CTSA initiative.

Answer:

The proposed reduction (\$3 million) to the CTSA program for FY 2015 primarily reflects redirected AIDS research funds to expand NIH support for research directed toward a cure for HIV. It does not reflect a reduced emphasis on the CTSA program. NCATS will continue to evolve the CTSA program, utilizing both the recommendations of the IOM report and the subsequent Working Group report.

3. The Clinical and Translational Science Awards (CTSA) program has primary responsibility for supporting translational research.
 - a. How does the rest of NIH fit into this picture?
 - b. Are CTSA's fully integrated into the entire NIH translational research plan?
 - c. Is there a specific mechanism to ensure alignment?

Answer:

Translational research continues to be conducted across NIH. NCATS was created to develop new and innovative tools and methods to improve how translational research is conducted and to disseminate that information across NIH and the research community. The activities of the CTSA program are one of the ways that NCATS is accomplishing this and they will continue to evolve to address this need.

The CTSA program has historically seen robust participation by the leadership of NIH I/Cs during the annual CTSA PI meeting each fall, which is planned to continue as the program moves forward. Additionally, both the Working Group and the recently formed CTSA steering committee include representation from the NIH ICs, which will help ensure that the program continues to evolve to be responsive to IC needs related to translational research.

19. Minority Health and Health Disparities

The administration claims to prioritize health disparities, yet the budget proposal level-funds the National Institute on Minority Health and Health Disparities while the NIH budget overall increases.

- Can you explain this apparent lack of support?

35

Answer:

The National Institute on Minority Health and Health Disparities (NIMHD) has a unique and critical role at the NIH as the focal point for conducting and coordinating research on minority health and health disparities, raising national awareness about the prevalence and impact of health disparities, and the dissemination of effective individual, community, and population-level interventions to reduce and ultimately eliminate health disparities. However, NIH has identified some other priorities, and given that funds are limited, has decided to put any additional funds available into trans-NIH efforts like the Brain Research through Advancing Innovative Neurotechnologies (BRAIN) Initiative, and Alzheimer's disease research, which are expected to yield health benefits to all populations.

- What initiatives are taking place at NIH to ensure a more robust pipeline of minority researchers?

Answer:

NIH investments in workforce diversity span the last 30+ years. These programs focus on individuals from underrepresented backgrounds, which include racial and ethnic minorities, persons with disabilities, and individuals from disadvantaged backgrounds (at the undergraduate and below level). Women are also underrepresented at the faculty and above level. Most recently and seminal to this effort, the NIH launched three inter-related initiatives to address issues of diversity in the NIH-funded biomedical workforce. These are NIH (1) Building Infrastructure Leading to Diversity (BUILD), (2) the National Research Mentoring Network (NRMN), and (3) the Coordinating and Evaluation Center (CEC). Programs such as BUILD, NRMN, and CEC support NIH's efforts to enhance the diversity of the biomedical research workforce. By utilizing the synergy between these programs, NIH can move forward to maximize the return on investment from the available resources.

- The BUILD initiative intends to test new, innovative approaches to recruitment, and training of scientists from diverse backgrounds. The emphasis is on development of culture-changing methods to motivate young scientists for careers in biomedical research and to enable them to thrive in the NIH-funded environment. It will support training at multiple career stages and promote faculty development at comparatively under-resourced institutions with a track record of producing and supporting students from backgrounds underrepresented in biomedical and behavioral research. This initiative recognizes the critical role that the faculty-student relationship plays and will provide salary offset and other mentor-promoting activities to enable outstanding mentors to work with students and train new mentors.
- The NRMN initiative is intended to augment local mentoring efforts for undergraduate students through junior faculty members by creating a national group of scientific leaders who are willing to serve as external mentors. The NIH intends to identify an entity that will engage and assemble multiple persons and/or professional organizations into a single, nationwide, consortium of mentors. The

NRMN will develop contemporary methods to facilitate networking between mentors and mentees but will also promote face-to-face experiences as needed. This initiative will address the standard wisdom that says that success depends on "who you know," by ensuring that contacts are made between mentees and mentors with shared interests and by facilitating subsequent interactions.

- The goal of the CEC initiative is to assess efficacy of the new approaches being developed via BUILD and the NRMN and to disseminate lessons learned across the community at large. It will help ensure optimal coordination of the BUILD and NRMN activities, minimize redundancy, and facilitate data tracking and analysis.

In addition to the Diversity Program Initiatives, in January 2014, Hannah Valentine, MD was appointed the first permanent Chief Officer for Scientific Workforce Diversity.

20. Tuberculosis (TB)

Question:

Dr. Fauci - Thank you for your commitment to research that will ultimately improve the current tuberculosis (TB) drug regimens and diagnostics.

Please describe how NIAID is working to improve TB treatments and diagnostics, and the potential impact of these improvements on public health.

Please also describe the partnership between NIH, the CDC, and other agencies to address TB and include details on how the agencies complement rather than duplicate one another's efforts.

Answer:

The National Institute of Allergy and Infectious Diseases (NIAID), lead component of the National Institutes of Health (NIH) for research on tuberculosis (TB), conducts and supports research to understand the basic biology of TB and to develop improved tools to prevent, rapidly diagnose, and safely and effectively treat TB. In particular, NIAID aims to discover novel TB drugs; to shorten the duration of TB therapy; to identify effective vaccines; and to improve diagnosis of drug-sensitive and drug-resistant TB. NIAID's basic and clinical research investments have contributed to the successful development of the GeneXpert MTB/RIF diagnostic test used worldwide to rapidly diagnose TB and drug-resistance to rifampicin, a commonly used TB drug. Globally, two-thirds of drug candidates and half of TB vaccine candidates currently in clinical development have received NIAID intramural or extramural support. The public health impact of these interventions could be profound in helping to lower the burden of TB worldwide.

NIAID has a longstanding commitment to improve diagnosis and treatment of TB. NIAID continues to support expansion of the GeneXpert diagnostic test to provide information about resistance to other important drugs used to treat TB. In addition, NIAID has supported studies, including one in collaboration with the Centers for Disease Control and Prevention (CDC), to evaluate the use of GeneXpert in TB-HIV co-infected individuals.

NIAID also works to identify and test improved TB treatment regimens, including shorter courses and combination drug therapies for difficult to treat infections such as drug-resistant TB and TB-HIV co-infection. NIAID scientists collaborate with international investigators on several studies of drug-resistant TB and clinical trials of novel TB treatments. An NIAID-led trial with South Korean collaborators recently found that the existing drug linezolid is effective against previously untreatable extensively drug-resistant TB. Based on these results, NIAID scientists are currently developing a clinical protocol to study a six-month regimen of linezolid combined with other clinical candidates in late-stage development for treatment of multi-drug resistant (MDR) TB. NIAID-funded scientists are partnering with the Global Alliance for TB Drug Development to compare four-month TB drug combinations against the standard six-month regimen. NIAID also is supporting a trial of an ultra-short one-month treatment regimen in TB-HIV co-infected individuals. In addition, NIAID recently restructured its HIV/AIDS Clinical Trials Networks to include studies on TB, with and without HIV co-infection, and to perform trials of improved treatment regimens for drug-resistant TB. One such trial will be the first to study two new drugs for MDR TB, bedaquiline and delamanid, together in combination treatment.

NIH participates with other federal agencies such as CDC and the United States Agency for International Development (USAID) in the Federal TB Task Force in order to coordinate TB research and control activities among member agencies. NIAID has the lead for basic and applied research and CDC has the lead for detecting and responding to emerging health threats and implementing advanced technologies to prevent disease. USAID works with countries worldwide to improve TB services and reduce the potential of drug resistant TB. These missions are distinct and complementary. The agencies work together, in collaboration with government and industry partners, to advance the common goal to remove TB as a threat to global public health.

21. Pediatric Preclinical Testing Program

Question:

Please provide a summary of how NIH and specifically NCI makes funding allocation decisions related to pediatric cancer. Further, please describe how much is being spent and what activities are on-going or planned in the FY 2015 request by IC to support a Pediatric Preclinical Testing Program.

Answer:

NCI supports a broad spectrum of cancer research. The research NCI oversees uses a wide variety of approaches and funding mechanisms, with several goals: improving our understanding of the causes and biological mechanisms of a large variety of cancers; preventing cancers; detecting and diagnosing all types of cancers; and treating cancers, as well as the symptoms and sequelae of cancers, more effectively. These efforts include studies of the basic aspects of cancer biology at the molecular and cellular levels, and applications of basic research discoveries toward successful detection, diagnosis, treatment, prevention, and control of cancers of all types, including pediatric cancers.

Pediatric cancer research projects, like all projects supported by the NCI, are subjected to rigorous review for quality and purpose by panels of expert reviewers, program staff, and external advisory groups. Decisions about individual grant applications selected for funding are based on a series of rigorous evaluations performed by scientific peers, NCI divisional program staff, and NCI Scientific Program Leaders, and are then subjected to final approval by the National Cancer Advisory Board. An emphasis on scientific merit and potential significance is maintained throughout the review process. All of these efforts are monitored annually through written progress reports and subjected to competitive peer review or terminated on a regular basis, generally between two to five years. Similar processes are used to oversee the representation of various types and costs of research in our portfolio.

The Pediatric Preclinical Testing Program (PPTP) is an NCI-supported effort which identifies new, more effective agents for treating childhood cancers through collaborations with more than 50 companies to evaluate more than 80 therapeutic agents. The primary goal of the PPTP is to develop data to help prioritize therapeutic agents for testing in pediatric clinical trials by providing a consistent and reproducible testing approach. Several PPTP-tested agents have moved into clinical trials through the NCI-supported Children's Oncology Group (COG) and the Pediatric Brain Tumor Consortium (PBTC). For example, selumetinib, an agent that targets a gene signaling pathway known as MEK, was shown to have high activity against a subset of Pediatric Low Grade Astrocytomas (PLGA) with a specific mutation in a gene called BRAF, which is located on the MEK pathway. A phase I trial building upon these results was developed by the PBTC, and in this trial (NCT01089101) patients with BRAF-mutated PLGAs responded to treatment by showing tumor shrinkage. The trial is now in a Phase II expansion. Additionally, a clinical trial was recently activated through the COG Phase I Consortium (NCT02116777) to evaluate a combination therapy identified by the PPTP as having activity against Ewing sarcoma. BMN 673, a compound that inhibits DNA repair, was combined with temozolomide, which causes DNA damage, and a study of the combination therapy has moved quickly from preclinical testing (began in October, 2012) to clinical evaluation (trial activated April, 2014). Additionally, three clinical trials building upon PPTP results are expected to be activated in the coming year by COG and the COG Phase I Consortium. The PPTP was recently approved for another five-year funding cycle. NCI expects to award \$2.7 million to PPTP sites in FY 2015; however, the actual amounts awarded will depend on NCI's FY 2015 appropriation. The PPTP will support critical preclinical research for pediatric cancers throughout the remainder of FY 2014, and we expect those activities to continue during the following five years, and to systematically evaluate novel agents for their potential utility against selected childhood cancers.

22. Advanced Consultation with the Extramural Community

Question:

The 2014 enacted appropriations requested NIH establish a mechanism to conduct advance consultations with the extramural research community prior to shifting the proportion of resources out of extramural research program to intramural. Please describe how this

mechanism is being established and what specific steps have been taken to date? Further, please describe how peer reviews in the study sections are provided the full information to ensure they understand the full scope in intramural activities that may relate directly or indirectly to their work during peer review.

Answer:

If an urgent need for additional investments in intramural research activities were to arise (such as required by a public health emergency or extraordinary research opportunity), it would be reviewed by the National Advisory Councils of the individual ICs and/or the Advisory Committee to the Director, or addressed through advice from the Director's Scientific Management Review Board, all of which include representatives from the extramural community.

In a few instances, NIH funding opportunities allow NIH intramural investigators to apply and their applications are evaluated through the extramural peer review process. If the applications are found to be meritorious, the award is made with intramural funds. The extramural research community is notified about funding opportunities available to intramural investigators by publication of the funding opportunity announcement in the NIH Guide to Grants and Contracts. For those few applications from intramural investigators, reviewers receive the same information as they do for other applications.

It is important to note that peer review study sections are directed to assess the scientific and technical merit, and potential impact, of the work proposed. Similarly, the National Advisory Council/Boards are directed to provide advice on the unique and promising nature of the science proposed, and not the research budgets. NIH program officers monitor ongoing research activities in their field, including those of intramural research laboratories, to identify new research opportunities as well as areas of scientific overlap, and advise their ICs about the best ways to invest in their research portfolios. Responsibility for the assessment of scientific and budgetary overlap, including overlap with intramural projects, is not the responsibility of peer review. Rather it is the responsibility of the IC Directors with assistance and counsel from their Program Directors.

23. Administrative Burden Reduction Workgroup

Please describe the process and link to where research institutes that desire to participate in the Administrative Burden Reduction Workgroup can get involved in this process with NIH to measure the burden with a goal of reducing the administrative burden on these organizations. Further, please provide a schedule of when the workgroup is expected to meet over the next year.

Answer:

There are three different groups working on reducing administrative burden on organizations that are the recipients of NIH grant awards:

The first is the Federal Demonstration Partnership (FDP), a cooperative initiative among 10 federal agencies and 119 institutional recipients of federal funds. Upcoming meeting dates are September 10-12, 2014; January 11-13, 2015; May 10-12, 2015; September 2-4, 2015. All meetings take place in Washington DC and the public can register for these meeting by visiting the FDP web site at http://sites.nationalacademies.org/PGA/fdp/PGA_051651#participation.

The second group is the Research Business Models (RBM) Working Group, an interagency working group of the Subcommittee on Social, Behavioral and Economic Sciences (SBE) of the Committee on Science (CoS) a chartered committee of the National Science and Technology Council (NSTC). RBM Working Group objectives include streamlining business models for the conduct of scientific research sponsored by the Federal government. The RBM solicits public comments and holds regional meetings to gather information related to the relationship between federal agencies and research performers on an *ad hoc* basis. There are no meetings presently scheduled for the public. RBM invites public input and comment through the Federal Register, in accordance with the Federal Register Act of 1935 (44 U.S.C. 1501, *et seq.*).

The third group is the NIH Scientific Management Review Board (SMRB), established pursuant to Sec. 401(e) of the PHS Act to advise HHS and NIH officials on the use of organizational authorities provided in the Act. At its meeting on May 7, 2014, the SMRB was charged with recommending ways to further optimize and streamline the process for reviewing, awarding, and managing NIH grants in a way that maximizes the time researchers can devote to research while still maintaining proper oversight. The SMRB will seek input from stakeholders and devote time to public comment at its public meetings, the next of which will be held on July 7–8, 2014. Meetings and information on signing up to make public comment will be announced in the Federal Register. Meeting agendas and future SMRB meeting dates will be posted on the SMRB web site (<http://smrb.od.nih.gov/>) as they become available.

24. NIH Clinical Center

The 2014 appropriations act directed NIH to include a non-add sub-line on the mechanism table with the NIH Clinical Center funding clearly reflected for all years. The FY 2015 budget request did not provide the requested information. Please re-submit the mechanism table with the requested information and include this information in future submissions.

Answer:

The requested information is provided below for FY 2013 and FY 2014. Due to the process for determining these funding contributions from Institute and Center (IC) intramural programs, there is not an estimate of the FY 2015 levels. Only the FY 2013 levels were available at the time of the FY 2015 budget request. In addition to the lack of data at the time of the request for two of the three years normally displayed, NIH does not consider this information necessary for the mechanism table. It represents less than 1.5 percent of total NIH funding, and only 12 percent of the listed Intramural Research line in the mechanism table.

Clinical Center Core Support ("School Tax")

| <u>IC</u> | <u>FY 2013</u> | <u>FY 2014</u> |
|-----------|----------------|----------------|
| NCI | \$112,439,123 | \$117,247,642 |
| NHLBI | 25,962,099 | 25,881,594 |
| NIDCR | 8,658,798 | 8,798,312 |
| NIDDK | 24,210,038 | 24,227,568 |
| NINDS | 21,249,721 | 21,266,646 |
| NIAID | 72,256,843 | 72,314,529 |
| NIGMS | 153,000 | 153,000 |
| NICHD | 25,522,232 | 25,683,813 |
| NEI | 9,835,374 | 9,843,208 |
| NIEHS | 19,921,352 | 19,912,395 |
| NIA | 8,370,000 | 8,670,000 |
| NIAMS | 7,338,515 | 7,340,988 |
| NIDCD | 5,475,781 | 5,329,040 |
| NIMH | 22,511,092 | 22,459,001 |
| NIDA | 8,391,000 | 8,360,000 |
| NIAAA | 6,573,094 | 6,581,162 |
| NINR | 905,891 | 1,050,158 |
| NHGRI | 14,136,060 | 14,085,799 |
| NIBIB | 1,638,961 | 1,539,485 |
| NCCAM | 1,112,952 | 1,113,837 |
| NIMHD | <u>941,074</u> | <u>941,823</u> |
| Total | \$397,603,000 | \$402,800,000 |

25. Consolidated Communications Activities

Please provide a detailed plan of all the actions taken or planned since the 2014 appropriations act was enacted to consolidate communications across NIH. Further, please update the report NIH provided last year with the costs NIH spend communications with FY 2013 actuals and FY 2014 estimates as part of the questions for the record, using the same definitions and format as used in last year's report.

Answer:

The Office of Communications and Public Liaison (OCPL) in the NIH Office of the Director has been working intensively with all the NIH Institute and Center and Office communications offices to identify solutions to further consolidate communications and increase collaboration across the agency to improve program effectiveness, streamline activities, avoid duplication of effort, and find cost savings. Currently, NIH is spending less than 1% of its budget on communications. As noted in the 2013 response, there are

numerous legislative mandates, including more than 55 provisions within the Public Health Service (PHS) Act, that authorize NIH to carry out science and health communications. Communicating research results to the public is an important step in research translation process and the public has come to rely on NIH's information resources, as reflected in the millions of visitors to the NIH web site.

OCPL is leading several trans-NIH efforts to address consolidation/streamlining/efficiencies. For example, it has just completed a workgroup on "print vs. digital" publication to identify cost-effective means of communications, while ensuring that all audiences are served. As a federal agency, we have unique opportunities and requirements to provide access for people who have disabilities and the needs created by challenges of literacy level and cultural and linguistic requirements. The workgroup considered issues of access, disability, 508 compliance, and audience need, and real savings and continuing costs.

OCPL also is leading trans-NIH workgroup on centralizing NIH communications infrastructural services, with three sub-working groups who are developing operational proposals to streamline 1) Warehousing/fulfillment activities: The group is looking at strategies for the most effective methods for shared information resource storage and fulfillment, including collaboration on contracts as they come up for renewal. 2) Exhibits (which is building on an already consolidated effort): The group is led by OCPL, the ICs, and the NIH Office of Human Resources to, whenever feasible, combine and strengthen NIH presence at scientific conferences, personnel recruitment, and share and save costs. They are exploring additional savings and are examining the pattern of exhibiting for optimal efficiency. Three examples of centralized exhibiting—instead of multiple ICs arranging separate exhibits at the Society for Neuroscience, the Biotechnology Industry Organization (BIO) International Convention, and the International AIDS conferences, the ICs coordinated a centralized exhibit presence, saving time, money, and reducing duplication of effort. Whenever possible, NIH partners with other government agencies to exhibit jointly and share costs. 3) Call and Contact Center Systems: The group is focused on analyzing the respective efforts of the ICs to provide health information to patients and public through telephone, mail, and the web and the potential consolidation of resources and potential impact of such consolidation on these services. All of the consolidation groups also are examining staffing and contracting, renewal dates, contract cycles, etc., that need to be understood for informed decision-making. In an effort to streamline contracting for communications services, OCPL and the ICs and the NIH Office of Acquisition and Logistics Management have been working toward the successful awarding of the Public Information and Communication Services (PICs) contract, an umbrella mechanism that provides a streamlined approach to securing public information and communication services across the agency. This mechanism has already been working to improve opportunities across ICs, and create resources for saving time and effort.

In addition to the over-arching, trans-NIH efforts toward streamlining communications across the agency, each Institute and Center and Office has initiated their own consolidation/cost savings efforts. The following are select examples from across NIH:

- NCI has continued to realize cost savings in several aspects of its communications functions. First, the NCI's Office of Communications and Education (OCE), Office of Media Relations, and Executive Secretary functions were consolidated into one NCI Office of Communications and Public Liaison (OCPL). In the three-office merger, staff numbers have been reduced through retirement, attrition, and reassignment of senior level staff. The number of federal staff devoted to this effort has been reduced from approximately 92 in FY 2013 to the current level of 73 in FY 2014. In addition, there has been an elimination of projects, reduction of contracts, and consolidation of efforts. This consolidation will result in greater efficiencies, less duplication of effort, and better coordination and consistency in NCI messaging. Expected savings would be seen in FY 2015. NCI has had a significant reduction in print inventory, with the increased use of e-books and more print material available online. This change has allowed for a 98% reduction in print expenditures. NCI's FY 2013 total communications budget was \$39,624,000, and the estimated FY 2014 OCPL (three consolidated offices) communications budget is \$39,082,048.
- NIDDK streamlined four separate communications contracts into a single task-order acquisition, using the Public Information and Communications Services (PICS) contract, managed centrally by the NIH Office of Communications and Public Liaison. The approach combines previously separate exhibit, evaluation, web content, message development, and partnership engagement efforts.
- NINDS initiated a number of activities to consolidate and coordinate efforts resulting in cost savings and/or greater efficiency in function. Examples include: taking part in the NIH central exhibit at major conferences, streamlining warehousing, and cutting the RMS budget for communications by 30% over the past 10 years.
- NHGRI has partnered with other ICs such as NICHD on program announcements to avoid duplication of effort and reduce costs, as well as participating in joint exhibit programs, and has played a key role in sharing information about social media as an efficient, effective means of communications.
- NICHD "retired" its Milk Matters communications outreach program, begun in 1998. Once the dairy industry began spending upwards of \$60 million each year on its "Got Milk?" (now "Milk Life") effort, the Institute's efforts were somewhat duplicative. As a result, NICHD communications saves annually approximately: \$100,000 in printing funds; \$15,000 in warehousing, storage, and fulfillment costs; \$5,000 in exhibiting/outreach costs; and \$5,000 in staff time; totaling \$125,000. Additionally, the Institute no longer prints copies of its scientific publications, such as reports, proceedings, and data updates, instead relying on the website and new media as the primary means of disseminating this information. Annually, this change saves: \$50,000 in printing costs; \$20,000 in warehousing, storage, and fulfillment; and \$2,500 in postage; totaling \$72,500.

- For the past several years, NIAID's communications office has actively taken steps to reduce annual operating costs through a combination of reducing reliance on outdated tools such as print publications and exhibits at conferences, reducing administrative support staff, and partnering with other ICs to take advantage of common communications systems.
- NIEHS and NCATS are working jointly with other federal agencies including FDA and EPA to communicate the Tox21 program activities, rather than each organization producing individual activities and products.
- Within NIEHS, communications and public education activities are carried out by six NIEHS programs and offices, over the last year communications activities have been consolidated and work roles coordinated in order to share staffing expertise and resources, and reduce the use of contractors.
- NCCAM has reduced its contract expenditures for its Clearinghouse activities. As the public increasingly relies on digital information, NCCAM has greatly reduced costs related to printing, mailing, and telephone inquiry response.
- In fiscal year 2014, NIBIB has continued to consolidate its website activities and has migrated to a new website content management system. This has resulted in savings for operating and maintaining the website and allows science writers and other content generators to take on the additional tasks for posting and updating content on the website. Since FY 2012, NIBIB's contract amount for public website activities was cut in half.
- NIA and NINDS, who have overlapping responsibility for funding and informing the public about research on "other dementias" (non-Alzheimer's), worked together to jointly develop and publish a series of information resources to make them more widely available in a more efficient way.
- NIAMS has reduced the cost of culturally-tailored health planners that are sent to underserved populations in all 50 states and five U.S. territories to help people who have conditions of the bones, joints, muscles, and skin.
- NHLBI is working with NLM to increase its content syndication to increase efficiency and reduce cost and duplication of effort.
- In partnership with HHS, NIH/NIDCR has begun syndicating its health information for use by public, private, and non-profit organizations. Content syndication allows organizations to subscribe to NIDCR digital content, at no charge, for use on their own websites, apps, widgets, and news feeds quickly and easily. This effort avoids duplication of effort and saves NIDCR the expense of creating its own content syndication system.

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- The Office of Extramural Research is saving money by using webinars and teleconferencing to present at professional society meetings, as well as expanding development of on-line videos for outreach.
- To maintain and update its public-facing website, NINR utilizes Drupal, an open source content management system. By using this platform, NINR staff can easily organize, manage, customize and publish content without the use of contractors—which reduces costs associated with web development and maintenance.
- NIAAA made contract modifications in FY 2014 that result in significant cost-savings that reduced NIAAA's obligation for this fiscal year by \$123,834 and reduced duplication of effort by partnering with NIDA on a videoconference series on a number of topics of mutual interest.

Below are the total communications budgets for FY 2013 and estimate for FY 2014. Many of these cost-saving and consolidation efforts may not be realized until FY 2015 or beyond. In addition, budgets are influenced by contracting cycles and legislative mandates such as the Language Access Plan, the Plain Language Act, and 508 compliance, each of which require substantial additional resources and effort.

- Total FY 2013: \$159,577,839
- Total FY 2014 (Estimate): \$159,500,000

26. Office of Directors Cost Breakout

Please provide the requested information from the 2014 enacted appropriations for each NIH IC Director's Office and the NIH Director's office for the total funding provided with the requested breakout on the cost of travel, personnel, performance bonus for each such office to be submitted in the response to the questions for the record. Further, we look forward to receiving this information in each future budget request.

Answer:

The FY 2014 Statement of Managers included the following request:

Institute & Center Office of Director Costs. -The NIH is expected to provide, in the fiscal year 2015 and future budget requests, a table that lists the total funding provided to the Director's Office of each IC and the NIH Director that breaks out the cost of travel, personnel, and performance bonuses by IC. The initial table should include the last three years of actual obligations, projections for the current year, and the fiscal year 2015 estimate.

The requested table is provided below. In order to use the most consistent and comparable data available, "Director's Office" was interpreted as the Immediate Office of the Director. This minimized the effect of different organizational structures among the Institutes and

Centers; there is substantial variation in the functions each IC includes in its broader Office of the Director organization. The information in the table could not be compiled in time for the FY 2015 budget request, and NIH does not consider it appropriate for inclusion in future budget requests. It represents less than 0.2 percent of total NIH funding, and the level of detail would be excessive for the Congressional Justification.

Questions for the record from Chairman Rogers

Question 1:

With 230,000 new cases of breast cancer diagnosed in 2013, along with nearly 40,000 deaths, breast cancer screening and early intervention continue to be critical tools in combating the spread of this disease. A host of advocacy organizations, health care providers and researchers support continued efforts at early detection through mammography. Additionally, new technologies are emerging in the field which may give health care providers clearer images, catching tumors that could be hidden in dense tissue and reducing false positives. What efforts is NCI making to increase mammographic imaging usage in the US?

Answer:

NCI conducts and supports high-quality research to understand and improve current screening strategies for breast cancer, to develop new screening techniques that will be more effective than current ones, and to evaluate whether screening strategies used to detect cancer will actually save lives. However, NCI does not directly provide screening services or guidelines for the use of such services. A few of our current activities that address breast cancer screening methods are described below.

Population-Based Research Optimizing Screening through Personalized Regimens (PROSPR) supports research to better understand how to improve the screening process (recruitment, screening, diagnosis, referral for treatment) for breast, colon, and cervical cancer. The overall aim is to develop multi-site, coordinated, transdisciplinary research to document the entire screening process and to evaluate and improve it. For example, the PROSPR research site at the University of Pennsylvania is one of three sites focused specifically on breast cancer screening, and this research aims to compare the false positive rates of 3D vs. 2D digital mammography, evaluate the ability of an imaging biomarker to reduce false positive rates, and test risk communication strategies.

Breast Cancer Surveillance Consortium (BCSC) is a resource for researchers who wish to examine the delivery and quality of breast cancer screening in the United States, including related patient outcomes. The BCSC is a collaborative network of seven mammography registries that link to pathology and/or tumor registries. The Consortium is led by a multidisciplinary team that includes radiologists, primary care clinicians, pathologists, epidemiologists, health services researchers, and biostatisticians. For example, NCI is currently supporting the Risk-Based Breast Cancer Screening in Community Settings project through the BCSC, and research underway through this project is described in more detail below in the answer to question 2 regarding new technologies. Planned BCSC analyses will assess trends in frequency and outcomes for interval cancers, those tumors that are missed by screening.

A recently developed inter-divisional NCI request for applications will focus on “overdiagnosis”---the detection of non-life threatening lesions and early tumors, a situation that often leads to inappropriate and potentially harmful treatment. This project will compare tumor biology and clinical aggressiveness with the method of detection, including breast imaging, and with the criteria used for diagnosis. Related projects evaluate factors associated with breast density, a strong breast cancer risk factor, and with mammographic features related to “tumor masking” and delayed detection.

Question 2:

What new technologies are available in the field of breast cancer screening?

Answer:

Researchers supported through the NCI BCSC are currently evaluating whether digital mammography plus Computer-Aided Detection improves mammography performance compared to digital mammography alone. These investigators are also evaluating community practice with advanced imaging technologies such as breast MRI, screening ultrasound, and digital breast tomosynthesis. Assessments of mammographic density in 3 versus 2 dimensions are also in progress. Additionally, as stated in the answer to question 4 on clinical trials to test mammographic technologies, NCI is providing an overview of a recently opened clinical trial that will evaluate an imaging approach using digital breast tomosynthesis combined with Tomographic Optical Breast Imaging.

NCI also funds the development and validation of 3D ultrasound technology to examine its potential for screening both high-risk women and the general population. Three-dimensional breast ultrasound technology, which does not require the radiation exposure and breast compression associated with mammography, permits high-quality imaging without danger or discomfort. This enables safe imaging of young women, offering possibilities to explore early events related to breast carcinogenesis. The currently widely used 2D breast ultrasound requires considerable expertise to acquire and interpret images. The 3D technologies, which typically yield higher-quality images with fewer false-positive results, may require less technical expertise, as image acquisition is partially automated. Computer-aided detection software is also being developed for breast cancer detection with 3D ultrasounds. In September 2012, the FDA approved the first breast ultrasound imaging system for dense breast tissue, for use in combination with standard mammography in women with dense breast tissue who have a negative mammogram and no symptoms of breast cancer.

NCI also funds studies of the biology of breast cancer and normal breast tissue to identify factors that might be useful for screening or that might affect risk for breast cancer. In projects partly funded through the sale of Breast Cancer Research Stamps, analysis of tissues and mammographic images are integrated to enhance the interpretation of radiological studies, including breast density. Biological research often leads to the identification of potential biomarkers for screening. This is research of critical importance in the field of breast cancer screening. NCI-funded research is exploring methods to identify women who are more likely to develop disease so that screening

resources can be concentrated on these women. For example, studies are being conducted to identify new breast cancer susceptibility genes and to further evaluate mutations in known breast cancer susceptibility genes.

In addition, NCI is funding research to identify biomarkers and approaches to complement current screening methods. Since cancers frequently elicit an immune response, researchers are attempting to define and use this response as a sensor to detect the presence and growth of breast cancers. In addition, NCI-funded projects seek to identify molecular signatures of breast cancer cells so that healthy tissues, benign growths, and cancerous areas can be distinguished.

Breast density has also been recognized as a potentially important biomarker of breast cancer risk, and NCI-funded investigators are asking how breast density may be related to breast cancer risk. Ongoing studies are also evaluating relationships between tamoxifen and breast density, given that the effectiveness of this agent in prevention and treatment has been associated with lower breast density. Density is also evaluated in other prevention studies using a range of agents, including aromatase inhibitors, low-dose tamoxifen, metformin, and polyphenon E. Preclinical and early translational research suggests that inflammation in the breast, particularly in the context of post-weaning re-modeling after pregnancy and in association with obesity, may be linked to increased breast cancer risk. Several projects explore the biology of these relationships and possibly interventions. Finally, in early work, breast milk is being assessed as a biospecimen for studying post-partum breast biology and possibly as a means of detecting or predicting risk of early onset cancers.

Question 3:

How do NCI and NIH evaluate what potential new technologies for imaging should be supported with clinical trials?

Answer:

All research efforts supported by the NCI are subjected to rigorous review for quality and purpose by expert peer reviewers, program staff, and advisory groups. Decisions about individual research projects selected for funding are based on a series of rigorous evaluations performed by scientific peers, NCI divisional program staff, and NCI Scientific Program Leaders, and then subjected to approval by the National Cancer Advisory Board. An emphasis on scientific merit, feasibility, and potential for application is maintained throughout the review process. All of these efforts are monitored annually through written progress reports and subjected to competitive peer review or terminated on a regular basis, generally between two to five years.

Additionally, the NCI National Clinical Trials Network relies upon the expertise and leadership of its Scientific Steering Committees (SCCs). SCCs are composed of leading cancer experts and advocates from outside the Institute as well as NCI senior investigators who meet regularly to increase the openness of the trial design and prioritization process, enhance patient advocate and community oncologist involvement in clinical trial design

and prioritization, and convene Clinical Trial Planning Meetings to identify critical questions, unmet needs, and key strategies. The SCCs include a Clinical Imaging Steering Committee, which serves as a forum for the extramural imaging and oncology communities to advise NCI about its investment in clinical trials of imaging methods. The Clinical Imaging Steering Committee is charged with identifying and promoting the “Best Science” in clinical oncology research by addressing the design and prioritization of large early and later phase clinical studies focused primarily on cancer imaging. In addition, CISC members provide valuable imaging expertise for other steering committees’ evaluations of therapeutic concepts and discussions that include an imaging component.

Question 4:

Please provide a list and the annual projected cost of current and planned clinical trials to test new mammographic technologies, such as tomosynthesis?

Answer:

NCI is supporting a new clinical trial, activated in February 2014: Digital Breast Tomosynthesis Guided Tomographic Optical Breast Imaging (TOBI). Currently, mammography is the most effective way of detecting early stage, non-palpable breast cancers. However, mammography reveals only the breast structure, and cannot determine its physiological state. The trial will evaluate Tomographic Optical Breast Imaging (TOBI) as an inexpensive technique that is non-invasive and does not use ionizing radiation. TOBI uses near infrared light to measure light passing through the breast, so that images of blood volume and hemoglobin oxygenation can be obtained. In this study, TOBI is combined with digital breast tomosynthesis (DBT, a form of 3D mammography), and the trial aims to determine whether TOBI-DBT combined images can be used to diagnose breast cancer with significantly improved sensitivity and specificity compared to DBT alone. The study is estimated to enroll 125 patients. NCI support for this trial in FY 2014 will be approximately \$465,000. This trial builds upon many years of NCI support---approximately \$1.9 million from FY2002-2006, and approximately \$2.3 million from FY2010-2013---to develop this combined imaging approach and prepare it for clinical evaluation.

In addition to this trial, NCI supports ongoing early phase feasibility trials evaluating a wide range of new technologies to improve mammography screening. In FY 2014, NCI will support 13 grants focused on new breast imaging technologies that have reached the early stages of clinical testing. The total FY 2014 support for these grants is approximately \$5.5 million. Technologies under study include improvements to diffusion-weighted magnetic resonance imaging (MRI); a no-compression breast ultrasound tomography scanner; and analytical software for breast tomosynthesis to test subtle features of breast density that may establish cancer risk.

Question 5:

On tomosynthesis technology, please advise the status of NCI plans to support a broad, randomized, clinical trial using tomosynthesis technology?

Answer:

Please see the answer to question 4, above, which provides an overview of a large, randomized trial of tomosynthesis currently underway with NCI support.

Representative Martha Roby

- 1.) Last year, Congress enacted into law the National Pediatric Research Network Act, which seeks to enhance our commitment to pediatric medical research by authorizing a network of multi-institution consortia pursuing scientific discovery and breakthroughs against the most devastating conditions impacting children today. What actions have NIH and NICHD taken to implement the network, and what actions are planned in the coming months?

Also, just recently Congress enacted legislation known as the Kids First Research Act, which reallocates funds that have subsidized our political conventions to pediatric research. This law speaks to our priorities as a nation. How does NIH plan to implement this law, and how could it be used to support the pediatric research network?

Answer:

The National Pediatric Research Network Act amended the Public Health Service Act to allow the Director of the National Institutes of Health (NIH), in consultation with the Director of the *Eunice Kennedy Shriver* National Institute of Child Health and Human Development (NICHD), to provide for the establishment of a pediatric research network. If such a network is established, the Director may utilize existing networks or centers already supported by NIH. If established, the law requires that an appropriate number of awards be made to those institutions conducting research on rare pediatric diseases.

To ensure that decisions are made with as complete information as possible, NICHD is in the process of finalizing a portfolio analysis of the existing pediatric research networks and centers programs supported by NIH. Most of the NIH Institutes and Centers (ICs) support a variety of pediatric research projects within their areas of expertise, many of which include research networks or centers. A conservative estimate is that NIH currently supports approximately 100 pediatric research networks and centers programs. Additional analysis is ongoing to determine what pediatric diseases and conditions are covered, and which networks and centers include rare pediatric diseases and conditions. Moreover, NICHD will engage its ongoing program evaluation and assessment efforts to identify ways to address current obstacles and emerging opportunities in pediatric research.

In addition, NICHD officials have met with a wide range of patient organizations and professional societies to discuss the law, its implications for pediatric research efforts, and to gauge what obstacles the law might have been intended to address. NICHD scientific and policy staff also have begun discussions about how to target these obstacles during the regular recompetition of currently funded pediatric research networks and centers programs through improved communication and data sharing across programs.

The Gabriela Miller Kids First Research Act authorizes a 10 Year Pediatric Research Initiative, to be funded through the NIH Common Fund, “only to the extent and in such amounts as are provided in advance in appropriation Acts.” If funds are appropriated for this purpose, the Common Fund would support initiatives in pediatric research consistent with the Common Fund mandate to support research in areas of emerging scientific opportunities, rising public health challenges, and knowledge gaps that deserve special emphasis; that would benefit from strategic coordination and planning across the NIH ICs; and that are designed to address specific goals and milestones. Common Fund programs enable research in many different diseases and conditions, including pediatric research, making data, tools, and resources widely available to the scientific research community.

- 2.) I want to raise the issue of diabetes. Over 11 percent of the population in Alabama has diabetes. I am concerned about them and their future, as well as the costly complications associated with this disease. Specifically, I would like to touch on diabetes-related kidney disease. The number of people with diabetes and kidney failure rose by 61 percent between 2000 and 2010. Also, as many as 50 percent of people with diabetes cannot achieve optimal blood sugar control so we must find ways to address this issue. Thanks to previous research, we know that tight glucose control can cut in half the onset of impaired kidney function, which leads to end stage renal disease. We all know the impact of ESRD has on the Medicare system so any relief there is meaningful to our U.S. economy.

Could you share what NIH is doing to help address diabetes and kidney disease?

Answer:

Controlling and preventing diabetes are the best approaches to preventing or minimizing its many health complications, including kidney disease. Diabetes—both type 1 and type 2—is the major cause of end-stage kidney failure. The landmark NIH-supported Diabetes Control and Complications Trial (DCCT) and its follow-up study, the Epidemiology of Diabetes Interventions and Complications (EDIC), demonstrated how critically important it is to control blood glucose levels early in the course of type 1 diabetes in order to reduce the likelihood of subsequent complications. DCCT participants who intensively controlled their blood glucose levels had significantly lower rates of eye, nerve, kidney, and cardiovascular complications than those who received standard care. This effect extended for many years after the study ended.

A second landmark NIH-supported clinical trial, the Diabetes Prevention Program (DPP), showed that an intensive lifestyle intervention designed to achieve modest weight loss through a combination of diet and exercise lowered type 2 diabetes rates by 58 percent, and that the generic diabetes medication metformin reduced diabetes rates by 31 percent, relative to placebo. The NIH-CDC National Diabetes Education Program (NDEP) sponsors a comprehensive prevention initiative, *Small Steps. Big Rewards. Prevent Type 2 Diabetes*, to translate the results of the DPP Study into public health practices. A follow-up study to the DPP, the DPP Outcomes Study (DPPOS), is assessing the long-term effects of interventions used in the Diabetes Prevention Program (DPP) on the development of type 2 diabetes and its complications. After 10 years of follow-up, DPPOS found that the lifestyle intervention continued to dramatically reduce the development of type 2 diabetes—and

consequently it complications—and also reduced cardiovascular risk factors. Additional NIH-supported research demonstrated the feasibility of substantially reducing the cost of the lifestyle intervention by delivering it to groups in community-based settings such as YMCAs. The CDC’s National Diabetes Prevention Program is based on these findings of NIH research.

Because there is no way to restore kidney function once it is lost, NIH research focuses on early detection of kidney disease and strategies to slow or prevent the progression of disease. The Chronic Renal Insufficiency Cohort (CRIC) Study, one of the largest and longest ongoing studies of CKD epidemiology in the U.S., is examining the natural history of kidney disease as well as the broad range of illnesses experienced by people with kidney disease. The NIH is supporting a study that aims to identify biomarkers that indicate a risk of progression of kidney disease. Research supported by the NIH has enhanced our understanding of the origin of scar tissue that is common in many forms of kidney disease, how it can impair kidney function, and how it might be prevented or treated. A new initiative, currently in development, will address challenges associated with growing nephrons, the kidneys’ basic filtering unit. The NIH also supports a number of studies that would be unlikely to be undertaken in the private sector, including pilot studies of novel therapies for kidney disease.

The NIH’s National Kidney Disease Education Program (NKDEP) is designed to raise awareness about the problem of kidney disease and steps that should be taken to treat CKD and prevent kidney failure. NKDEP represents a major educational outreach effort to patients, physicians, and the public. Efforts are underway to determine effective ways to translate advances in kidney disease prevention into improvements in patient care.

3. Question:

There is a lot of talk about translational research—converting promising early stage research into advanced development and eventually an actual product that can help save lives. Nowhere is that more critical for the federal government than in biodefense research and development – meeting the government’s need for medical products to protect Americans from national security threats such as smallpox and anthrax.

The concerns I have heard are early research investments in biodefense are based on investigator initiated research rather than focused on specific product goals for identified threats; the transition from early to advanced development is fragmented, and; the US government funding profile is more heavily research oriented – meaning funds are weighted towards early research rather than advanced development – when compared to industry ratios.

1. How does NIH target biodefense research funds to develop medical countermeasures against identified national security threats?

Answer:

The National Institutes of Health (NIH) supports research toward the development of medical countermeasures against biodefense threats such as anthrax, smallpox, plague,

influenza, and exposure to chemical or radiological threats. The National Institute of Allergy and Infectious Diseases (NIAID), the lead component of the NIH for biodefense research, conducts and supports a robust portfolio of basic research on microbiology and immunology to understand potential threats; applied research to develop candidate medical countermeasures; and early-stage clinical research to evaluate candidate diagnostics, therapeutics, and vaccines. NIAID coordinates with its Public Health Emergency Medical Countermeasures Enterprise (PHEMCE) partners, such as the Biomedical Advanced Research and Development Authority (BARDA) and Food and Drug Administration (FDA), and industry to ensure that the results of NIAID-supported research can be translated rapidly into safe and effective high-priority medical countermeasures. NIH biodefense research is guided by strategic plans and research agendas and is aligned with emerging public health needs and priorities identified by the PHEMCE.

NIH's basic research programs characterize the fundamental scientific and medical aspects of potential threat agents. This knowledge is necessary to shape current and future medical countermeasures development and to inform threat characterization conducted by the Department of Homeland Security. Basic research is especially important in cases of threat agents for which there are no currently identified countermeasures. Basic biological knowledge is critical to uncover potential diagnostics, therapeutics, or vaccines for known threats as well as newly emerging or currently unidentified threats. Investigator-initiated research generates important ideas that significantly contribute to the development of medical countermeasures. To further promote research in high-priority scientific areas such as biodefense, NIAID issues targeted initiatives. For example, NIAID recently solicited applications for new Centers of Excellence for Translational Research. The Centers facilitate interdisciplinary translational research to generate, validate, and advance medical countermeasures for emerging and re-emerging infectious diseases, including high-priority agents with bioterrorism potential.

Biodefense research supported by NIAID has resulted in the recent successful advancement of a number of high-priority medical countermeasures. For example, NIAID supported early-stage development of a next-generation smallpox vaccine that would be suitable for use by special populations for whom the currently licensed smallpox vaccine is contraindicated. NIAID has transitioned the next-generation smallpox vaccine to BARDA for advanced development. Additional products recently transitioned from NIAID to BARDA include therapies for anthrax and pandemic influenza as well as a next-generation anthrax vaccine. NIAID has worked closely with FDA to conduct studies that supported the first antibiotic approvals for pneumonic plague under the FDA's animal rule. NIAID also collaborated with the Centers for Disease Control and Prevention, FDA, and BARDA to rapidly develop a vaccine for 2009 H1N1 pandemic influenza.

2. Is the ratio of early research to advanced development funding appropriate for improving our chances of getting products fully developed and licensed? On what basis does HHS justify allocating 80-90% of biodefense funds toward basic

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research at NIAID, whereas at DOD the equivalent expenditures are only in the high 60% range?

Answer:

The National Institute of Allergy and Infectious Diseases (NIAID), the lead component of the National Institutes of Health (NIH) for biodefense research, conducts and supports basic, applied, and clinical research on biological, chemical, and radiological/nuclear threats to understand these threats and to identify potential medical countermeasures against them. Fundamental research supported by NIH lays the groundwork for advanced development of medical countermeasures in collaboration with government and industry partners.

NIH has transitioned to the Biomedical Advanced Research and Development Authority for advanced development candidate medical countermeasures against chemical nerve agents, radiation exposure, anthrax, smallpox, pandemic influenza, and others. In addition, NIAID has contributed to the development of broad spectrum antimicrobial therapies for tularemia and plague and broad-spectrum antiviral agents for poxviruses and Ebola virus. NIAID also provided support for basic, applied, and clinical research, including a Phase I clinical trial, to advance the development of a medical countermeasure to treat ingested and aerosolized botulinum toxin A. From Fiscal Year (FY) 2009 to FY 2013, basic research programs comprised 50 to 55 percent of NIAID's biodefense research funding. During this period, the remainder of NIAID's biodefense research funding was allocated to applied and early-stage clinical research to develop and evaluate drugs, diagnostics, and vaccines.

4. Question:

NIH recently joined with 10 pharmaceutical companies to work on development of new therapies for Alzheimer's disease, Type 2 Diabetes, rheumatoid arthritis and lupus. This initiative, known as Accelerating Medicines Partnership (AMP) recognizes that progress toward new therapies for common chronic diseases demands large-scale collaborative efforts. It is my understanding that schizophrenia – a severe and disabling brain disorder that creates significant public health costs and enormous demands on family caregivers – was considered, but later removed from the AMP list.

Why was schizophrenia excluded from the final list of initial AMP collaborative studies?

Will NIH give consideration to include schizophrenia in the next round of AMP projects?

Answer:

The Accelerating Medicines Partnership (AMP) is a unique type of public-private partnership, and the success of its projects is dependent on strong support from all of the members of AMP: NIH, FDA, nonprofit organizations, and biopharmaceutical companies. AMP was just launched in April, and as noted, is beginning with three specific projects. For each project, the AMP partners have agreed that there is a scientific opportunity that is amenable to a public-private partnership approach and for which industry was willing to commit considerable funds and other resources. While schizophrenia was not chosen as one

of the initial projects, the AMP partners intend to consider other projects in the future, and it is certainly possible that schizophrenia will be considered again.

NIH staff are currently working to maximize the likelihood of success of the first projects. The policies and practices developed in the initial AMP projects should also benefit future AMP projects. Strong NIH support for research on schizophrenia continues, with FY 2013 research funding at \$232 million, and an estimated increase in FY 2014 to \$236 million. NIH is supporting research to integrate new knowledge of molecular, genetic, and epigenetic mechanisms, in order to better understand the complex interplay of genetic, developmental, and environmental contributors to schizophrenia. Moving forward, NIH plans to pursue novel treatments using pharmacological, psychosocial, neurological, and rehabilitative methods during the prodromal, early, and chronic stages of illness.

5. Question:

One of the important investments NIH has made and continues to make is in rare and orphan disease research. Duchenne Muscular Dystrophy is one of the diseases in which NIH has made investments, and the resulting research has been enormously helpful in finding effective treatments. The good news is we are on the cusp of the first approved treatment for Duchenne. Building on NIH's research, and now based on over 120 weeks of data, there have been no adverse effects to patients and strong signs of efficacy.

I believe that NIH's success in this and other research is directly related to its use of every authority at its disposal to invest in these rare diseases. It has clearly yielded life-saving results in other cases.

1. Using existing authorities across agencies, what can we do better to realize the full potential of our investments in rare and orphan diseases, "from bench to bedside"?

Answer:

The full potential of investments in rare diseases research can be realized by increasing efficiencies in the "bench to bedside" process, and the National Center for Advancing Translational Sciences (NCATS) is striving to do just that. NCATS was established to get more treatments to more people more quickly. We now have the knowledge needed to develop treatments for many of the more than 6000 diseases (mostly rare, including many cancers) that have no FDA-approved treatments. Currently, only a handful of new treatments for previously untreatable diseases are approved each year. NCATS is directly addressing this problem, developing new technologies that will greatly accelerate the process of developing and deploying new treatments, and actively disseminating them to everyone that does translational research.

NCATS conducts research on rare and neglected diseases. The NCATS' Office of Rare Diseases Research (ORDR) coordinates research on rare diseases to serve the needs of patients, health care providers, patient advocacy groups and scientific communities involved in efforts to improve the lives of individuals with rare diseases.

The NCATS' Therapeutics for Rare and Neglected Diseases (TRND) program speeds the development of new drugs for rare and neglected diseases by developing collaborations among NIH and academic scientists, nonprofit organizations, and pharmaceutical and biotechnology companies working on rare and neglected illnesses. The work of TRND includes the discovery, optimization and preclinical testing of therapies, with the goal to generate sufficient quality data to support successful Investigational New Drug (IND) applications to the U.S. Food and Drug Administration (FDA) and first-in-human studies as necessary. Therapeutic clinical candidates that reach this stage should be attractive to biotechnology and pharmaceutical companies to take into clinical development.

One of the projects being worked on by TRND is on Duchenne muscular dystrophy (DMD). Current therapy, glucocorticoid steroid injection, lessens the severity of symptoms but does not treat the muscle-specific effects of DMD, and the side effects, such as bone fragility and stunted growth, can be serious. TRND researchers, working with the support of a pharmaceutical company, a philanthropy, the DOD, and a patient group, tested a lead compound and are working to submit the promising results to the FDA for approval to initiate first-in-human trials in DMD.

Another NCATS' program, the NCATS' Discovering New Therapeutic Uses for Existing Molecules program, is also working on DMD. This program speeds the drug development process by matching academic research groups with previously developed compounds from industry. Researchers at the Kennedy Krieger Institute in Baltimore and the University of Washington in Seattle are testing an oral, investigational drug developed by Sanofi for safety and effectiveness in a mouse muscular dystrophy model, which if successful will be followed by testing for safety and ideal dose level in DMD patients and then a multisite clinical trial to provide information on its potential benefit to DMD patients.

2. What additional authorities, if any, would help more closely connect the steps between research and approval for NIH and the other agencies involved in treatments for rare and orphan diseases?

Answer:

NCATS was created to transform the translational science process so that new treatments and cures for disease can be delivered to patients faster. Translational sciences comprise the process of turning observations in the laboratory and clinic into effective interventions that improve the health of individuals and the public—from diagnostics and therapeutics to medical procedures and behavioral changes.

NCATS already is working closely with other agencies that are part of the research and approval process. For example, NCATS' Tissue Chip for Drug Screening program is a collaboration with the Defense Advanced Research Projects Agency and FDA to develop 3-D human tissue chips that accurately model the structure and function of human organs, such as the lung, liver and heart. These devices will enable researchers to predict harmful health effects of new drugs more accurately, thus addressing one of the main reasons that drug studies so often fail.

As another example, Tox21 is a unique collaboration among NCATS, the National Institute of Environmental Health Sciences, the U.S. Environmental Protection Agency, and the FDA, aimed at developing better methods of assessing the potential toxicity of drugs and environmental chemicals. The initiative adapted high-throughput screening technologies developed in NCATS' Chemical Probe Development program for use in toxicology screening. Tox21 is measuring the effects of more than 10,000 different drugs and chemicals on a wide variety of cellular pathways and functions that may lead to adverse health effects. These data are being used to identify chemicals in need of more in-depth study, develop computational programs that will better predict toxicity of new drugs and chemicals, and improve the efficiency and accuracy of new drug and chemical development. All Tox21 data are being made freely available in public databases to empower all researchers to create predictive algorithms for potential toxicity of chemicals and drugs.

3. As you know, kids with Duchenne Muscular Dystrophy are diagnosed early in life and then live their short lives with the daily and progressive loss of muscle function. The disease has no approved treatment, and invariably involves loss of mobility leading to death by the teens or early 20s.

While NIH research has shed tremendous light on Duchenne Muscular Dystrophy, there are comparatively few clinicians who are Duchenne experts because of the rare nature of this disease. We seem to be experiencing a disconnect when a treatment is ready for review and approval, in that the scientists at FDA don't seem to understand the underlying condition in ways that the top researchers and clinicians do.

Does NIH share scientists and resources with the FDA to ensure that we expedite approval of treatments by reducing gaps in the understanding of the science of conditions like Duchenne? If NIH does not do this, what changes need to be made to facilitate this kind of information and resource-sharing?

Answer:

NIH and FDA staff regularly interact and share information relevant to therapy development and approval for many diseases and disorders, including Duchenne muscular dystrophy (DMD). A memorandum of Understanding (MOU) between the Food and Drug Administration/Center for Biologics Evaluation and Research (FDA/CBER) and the National Institutes of Health/National Institute of Neurological Disorders and Stroke (NIH/NINDS) provides a framework for coordination and collaboration between these two agencies. As part of this agreement, staff from the two agencies hold periodic joint meetings to promote better communication and understanding of regulations, policies, and statutory responsibilities, and to serve as a forum for questions and problems. General issues related to the translation of research findings into disease therapies, as well as specific topics such as stem cell research and gene therapy are discussed.

Specific to DMD, an FDA representative is a member of the Muscular Dystrophy Coordinating Committee, which includes representatives from other agencies involved in muscular dystrophy research as well as members of the muscular dystrophy patient and advocacy community. A recent workshop, jointly sponsored by the Muscular Dystrophy Association and NINDS on *Best Practices for Gene Therapy Programs* included FDA participants who discussed FDA regulation of gene therapies as well as issues in the regulatory process. DMD was one of the specific diseases discussed within this context. In addition, FDA and NIH co-organized a workshop in 2010, NIH/FDA Conference on Antisense Oligonucleotide Therapies in Neuromuscular Disease, that specifically addressed issues on development and approval of the class of drugs being testing in exon skipping clinical trials in DMD.

Aside from workshops and more formal interactions, NINDS staff – some of whom have prior FDA experience themselves – regularly interact with the FDA on scientific issues relevant to their specific portfolios.

6. Question:

1. In the current economic climate, how do you plan to continue your support of the extramural research community and the critical role they play in scientific advancement?

Answer:

The majority of NIH's annual budget remains targeted for extramural grants-in-aid and R&D contract mechanisms. In FY 2013, extramural grants and R&D contracts awarded to universities, companies, and other recipients reached nearly \$23.57 billion, or slightly under 81 percent of overall NIH budget authority. NIH plans to continue FY 2013 investment levels for FY 2014, with a projected 81 percent share of appropriated resources identified for extramural research. Approximately \$24.36 billion is allocated to Research Project grants, Research Center grants, Other Research grants and R&D Contract mechanisms of the total \$30.14 billion budget authority received by NIH in FY 2014. In the FY 2015 President's Budget, the NIH request reflects a slight increase in funding compared to the FY 2014 estimate.

2. Can you tell me more about the New Common Fund and share details on how the \$30M would be spent?

Answer:

Begun in 2004 as the NIH Roadmap and re-named in the 2006 NIH Reform Act, the NIH Common Fund supports research in areas of emerging scientific opportunities, rising public health challenges, and knowledge gaps that deserve special emphasis; that would benefit from strategic coordination and planning across the NIH Institutes and Centers (ICs); and that are designed to address specific goals and milestones. To this end, Common Fund programs tackle major challenges in biomedical research that affect many diseases or conditions or that broadly relate to human health. Collectively, Common Fund programs address challenges and opportunities that have been identified as being among the highest priority for the scientific research community and the NIH. The Common Fund currently

supports close to 30 programs, with an FY 2014 Enacted budget of approximately \$533 million. The FY 2015 President's Budget request of approximately \$583 million will support ongoing initiatives within current Common Fund programs, and will expand certain other activities, such as the Big Data to Knowledge (BD2K) program, the Building Infrastructure Leading to Diversity (BUILD) initiative within the Enhancing the Diversity of the NIH-Funded Workforce program, and the Undiagnosed Diseases Network. It will also support the launch of several new programs. Programs under development for FY 2015 include 4D Nucleome, which aims to study the relationship between nuclear architecture and gene expression/cellular function over time, and Glycoscience, which aims to enable the analysis of sugar compounds that are attached to most proteins and play critical roles in health and disease. An additional program for FY 2015 is the Stimulating Peripheral Activity to Relieve Conditions (SPARC) program, which aims to develop precise and effective methods to stimulate the peripheral, autonomic, and enteric nervous systems to treat multiple diseases and conditions.

Question:

7. Stroke places a large burden on my State of Alabama. Not only does Alabama lay in the "Stroke Belt," we have the deadly distinction of having the highest age-adjusted stroke death rate in the US. I understand that that the cost of stroke may become worse in future years. For example, a recent study projects that the direct costs will triple by the year 2030. Can you please tell me how NIH is addressing this issue, including preventative measures?

Answer:

NIH recognizes the high burden that stroke places on communities across the country, as well as the disproportionately higher burden experienced in some population groups. NIH invests in research that aims to better understand why these differences exist and to develop effective strategies for combating these disparities.

The Reasons for Geographic and Racial Differences in Stroke (REGARDS) study, which is led by investigators at the University of Alabama in Birmingham, compares stroke rates and stroke risk factors in the Stroke Belt to other U.S. regions. REGARDS includes over 30,000 persons and has improved our understanding of factors that contribute to stroke risk and how race and geographic location can influence those risk factors. Other large-scale epidemiology studies such as the Northern Manhattan Study, the Greater Cincinnati Northern Kentucky Stroke Study, and the Brain Attack Surveillance in Corpus Christi study, are also contributing to a wealth of knowledge about stroke risk and burden in different subpopulations.

The National Institute on Neurological Disorders and Stroke (NINDS) has also been proactive in enaging investigators to develop innovative methods that can reduce health disparities. The NINDS supports four regional programs to develop and test culturally tailored interventions that address some of the major known contributors to stroke disparities. Projects within the programs include clinical trials of interventions to improve blood pressure control, effectiveness studies testing novel approaches to delivery of care such as mobile health technologies and transitional care, and observational studies to

identify and quantify temporal trends in risk factors. These programs have built partnerships with communities and stakeholders such as Kaiser Permanente and the American Heart Association's Get With The Guidelines program, and include training/education, community outreach, as well as plans for scaling and disseminating successful interventions more broadly.

In order to translate new knowledge from population studies into interventions that address this problem, the NINDS, in partnership with the NIH Office of Disease Prevention, held a workshop in November of 2013 to inform development of solutions to stroke disparities and reveal areas where additional research is needed. Leaders from multiple Federal agencies met with investigators and representatives from multiple communities to address how a greater level of stakeholder collaboration could make progress on this complex issue, which has socioeconomic, biologic, environmental, and policy underpinnings and implications. Recommendations from the workshop are being finalized and will identify primary and secondary prevention strategies to reduce disparities in stroke.

Hypertension is one of the most important risk factors for stroke, and disparities in hypertension prevalence and control contribute to the higher burden of stroke in minority populations. Uncontrolled hypertension can also lead to disease of the small vessels in the brain, which is much more prevalent in minority populations, and can cause stroke as well as cognitive impairment and dementia. The NINDS and multiple other NIH Institutes are partnering with the Patient Centered Outcomes Research Institute to develop an initiative that addresses health disparities in hypertension, with the ultimate goal of preventing stroke, heart disease, and vascular contributions to dementia.

1. Not only does stroke place a large burden on Alabama, total cardiovascular disease does too. My state ranks 51 out of 52 highest in our Nation for age-adjusted death rates for cardiovascular disease—only Mississippi outranks my State. Unfortunately, cardiovascular disease remains the No. 1 and most costly killer in the US. An estimated 84 million adults in the United States live with some form of cardiovascular disease. Can you please tell me how NIH is addressing cardiovascular disease, including preventive measures?

Answer:

The National Heart, Lung, and Blood Institute (NHLBI) at the NIH has a proud history of supporting research and awareness of cardiovascular disease (CVD). While CVD remains the leading cause of death for both men and women in the U.S., NHLBI-supported research has contributed to a 70 percent decline in CVD deaths from 1950 to 2008. This is due in no small part to studies like the Framingham Heart Study that first identified the CVD risk factors now addressed in routine physicals. This finding led to basic research on cholesterol metabolism that won NIH-funded researchers Michael S. Brown and Joseph L. Goldstein the Nobel Prize in 1985, a discovery that set the stage for the development of statin drugs and the development of the Dietary Approaches to Stop Hypertension (DASH) diet, which was shown in clinical trials to lower blood pressure, rivaling the effects of antihypertensive drugs.

While there have been overall declines in CVD deaths, these have not been borne by every segment of society. CVD mortality continues to be highest among African Americans, for example. NHLBI continues to support research to understand these disparities, such as the Jackson Heart Study (JHS), which has and continues to provide important insights into CVD risk factors in this population. A recent JHS study showed that the gene *APOL1*, which is known to contribute to chronic kidney disease, also increases CVD risk in African Americans. Findings such as these can improve prevention efforts, tailor treatment regimens, and may open the door to new targets for medications development to prevent kidney disease or heart failure.

NHLBI also continues to fund a broad portfolio of CVD prevention research from basic science discoveries such as uncovering the pathways that affect cholesterol, like the recent discovery of a mutation in the gene *PCSK9* that has led to the rapid advancement of *PCSK9* inhibitors as the next generation of cholesterol lowering drugs, to trials that test behavioral interventions to increase adherence to weight loss recommendations, and studies that test the most effective ways for controlling risk factors, particularly in minority populations.

Scientists are also investigating ways to identify new drug targets and test potential new medications more efficiently. For example, researchers are using adult stem cells to create heart cells that can be used as platforms (i.e., high throughput screening) to screen many different novel drugs to determine toxicities and promise for treatment, expanding the search for therapies and doing so with greater efficiency.

Finally, the NHLBI is committed to ensuring that research results reach the public. NHLBI has been a leader in raising awareness about heart disease and what individuals can do to lower their risk through campaigns such as The Heart Truth® for women and Million Hearts®, focused on prevention of heart disease and stroke. NHLBI also partners with professional societies to provide physicians the latest scientific evidence to inform clinical practice. In 2013, NHLBI partnered with the American Heart Association (AHA), the American College of Cardiology (ACC), and other professional societies to provide the systematic evidence reviews that led to the release of four clinical practice guidelines in primary cardiovascular prevention focused on lifestyle, risk assessment, cholesterol, and obesity.

A recent study exemplifies the importance of CVD research and how it can be used to improve the public health. The NIH-supported Women's Health Initiative, found—contrary to conventional wisdom—that the widely-prescribed estrogen/progestin postmenopausal hormone therapy not only failed to protect women from heart disease but also increased their risk of developing invasive breast cancer. Dissemination of these findings resulted in a dramatic reduction in prescriptions for combined hormone therapy, 76,000 fewer cases of CVD, and 126,000 fewer breast cancer cases, saving \$35.2 billion in direct medical costs. In fact, the federal investment in WHI resulted in a return of approximately \$140 in net economic value for each dollar invested in the trial, improving both the health and wealth of the nation.

Question for the Record from: Rep. Chris Stewart

Dr. Collins, at our public witness hearing on March 26th, the subcommittee heard from Dr. Vivian Lee about the Utah Genome Project, which is utilizing the Utah Population Database to discover the genetic causes of disease, particularly cancers. As you know, the Utah Genome Project was instrumental in identifying the BRCA 1 and 2 genes that cause breast cancer, in addition to a gene linked to 80 percent of colon cancers. I know this area of research is particularly important to you since you are personally responsible for much of the ground-breaking science that led to the ability to map the human genome. NIH has spent vast sums of money on the research that has gotten us to where we are today -- paving the way for the work now being done in Utah and elsewhere to identify the defective genes that cause particular diseases. How are you prioritizing funding for research such as the Utah Genome Project that is leveraging the tremendous investments NIH has made on DNA sequencing?

Answer:

The Utah Genome Project (UGP) is an initiative by the University of Utah to discover novel disease-causing genes and develop genomic-based diagnostic tools for clinical use. While NIH does provide funding to the University for research, the University of Utah provides its own funding for the UGP.

Research endeavors, such as the UGP, are now possible thanks in large part to the overwhelming success of the Advanced DNA Sequencing Technology Program of the National Human Genome Research Institute (NHGRI)³⁰. This program has contributed to reducing the cost of DNA sequencing by more than five orders of magnitude (i.e., 100,000-fold) since the end of the Human Genome Project in 2003. This, coupled with the investment in research data repositories such as the NIH's National Center for Biotechnology Information, has enabled many more investigators throughout the biomedical research enterprise to incorporate genomic analyses into their research investigating the causes of human disease.

NIH is supporting research to discover how the genome contributes to both rare and common diseases. First, more than 25 million Americans suffer from rare diseases, caused (for the most part) by a mutation in a single gene; these are also known as Mendelian diseases. Some of these may only affect a handful of families worldwide, others may afflict more than a hundred thousand individuals; while individually rare, these diseases are cumulatively more common than cancer. While the genomic bases for more than ~5000 rare diseases have already been established, the causal gene(s) for many additional rare diseases remains to be identified. To this end, NHGRI's Centers for Mendelian Genomics Program aims to uncover the genomic bases for the ~3000 Mendelian diseases for which a causal gene has yet to be discovered.

³⁰ Nature 507, 294–295 (20 March 2014) <http://www.nature.com/news/technology-the-1-000-genome-1.14901>

NIH is also exploring the genomic underpinning of rare diseases through its Undiagnosed Diseases Program (UDP), which seeks to provide answers to patients with mysterious conditions that have eluded diagnosis. Such has been the success of the program that it has led to the establishment of a new NIH Common Fund initiative, the Undiagnosed Diseases Network, which will replicate the UDP approach taken within the NIH Clinical Center in several medical centers across the United States.

NHGRI and many other Institutes and Centers across NIH are also funding research to elucidate the genomic bases of more common diseases. Common diseases, such as cancer, cardiovascular disease, or asthma often have a heritable component (i.e., these diseases run in families), but genetic and genomic factors only partly explain those conditions. Prior to the advances enabled by NIH's investment in DNA sequencing, it was difficult to establish the contributions of any specific variation in an individual's DNA code because of the complicated interplay between genetic and environmental factors.

Examples of research to accelerate the identification of genomic contributions to common diseases include The Cancer Genome Atlas (TCGA) and the Alzheimer's Disease Sequencing Project (ADSP). TCGA is a collaboration between NHGRI and the National Cancer Institute; since 2006, TCGA has analyzed the mutated genome sequences of more than 8000 tumor specimens from 27 different types of cancer, revealing many new insights into these diseases. Although in its final phase, TCGA is currently expanding to 10,000 tumor samples. The ASDP is a collaboration with the National Institute on Aging (NIA) that brings NHGRI's three flagship genome sequencing centers to bear to sequence the genomes of 582 research participants from 111 families within well-studied Alzheimer's disease patient cohorts. This partnership between NHGRI and NIA will be immensely helpful in establishing how best to use genome sequencing to advance the scientific understanding of this complex disease that affects as many as five million Americans aged 65 and older. This work is consistent with Congress' recognition of the severity of this neurodegenerative disease through the passage of the National Alzheimer's Project Act in 2011.

NHGRI is also funding research to use the knowledge generated from the projects described above—and others—to improve clinical care by taking genomic medicine out for a 'test drive' in the clinical setting. Pilot programs like the Implementing Genomics Into Clinical Practice (IGNITE) Network and the Clinical Sequencing Exploratory Research (CSER) Program are at the vanguard of exploring the utility of genomic medicine. Although these programs are primarily aimed at learning how best to incorporate genomic analyses into the clinic, they are also providing novel and important discoveries about genomic contributions to specific diseases.

QUESTIONS FROM REPRESENTATIVE ROSA L. DELAURO

Prescription Drug Abuse

Question for Dr. Collins

Question:

Dr. Collins, as you know, prescription drug abuse is a major public health threat, specifically the abuse of opioids and painkillers. Overdose deaths from prescription painkillers have quadrupled since 1999 and now outnumber those from heroin and cocaine combine. In my home state of Connecticut, one of every five teenagers has abused prescription opioids at some point in their life, and in many states – including Connecticut – opioid overdose deaths are outpacing deaths from motor vehicle accidents.

1. What is the NIH doing to address research into the abuse of and addiction to these substances? Specifically, we would be interested to hear about the work being done by the National Institute on Drug Abuse, and what they are doing not only in research, but in the translation of that research into practice for use by public health professionals?

Answer:

It is important to note that opioid medications are among the most effective interventions we currently have for managing severe pain. Unfortunately, these drugs not *only* inhibit pain centers in the brain but also activate brain reward regions—which is why they are abused and why they can be so addictive. So, NIDA's multipronged activities in this area represent a strategic response to the unique challenge of preventing opioid abuse and addiction while safeguarding the value for opioid medications in managing severe pain, which if untreated, is terribly debilitating.

Consistent with this strategic stance, our three major research goals relate to the safe management of pain, the prevention of overdose deaths, and the treatment of opioid addiction.

- 1) *Research related to Safe Management of Pain.* We still don't know enough about the risk for addiction among chronic pain patients. So, apart from basic research to understand how pain itself influences the addictive potential of prescription opioids, top research priorities include development of (I) non-opioid-based analgesics that are not addictive; (II) new formulations and delivery systems for opioid medications with less risk for diversion or abuse; and (III) non-medication strategies for managing pain, such as transcranial magnetic or electrical brain stimulation.
- 2) *Research related to treatment of opioid addiction.* Medication assisted therapies such as methadone, buprenorphine, and naltrexone, are effective in treating opioid addiction and in reducing deaths from overdoses. But these medications are used in less than one third of patients who need them. NIDA continues to conduct research on the nature of these

barriers to help inform strategies to enhance adoption of these treatments. In parallel, research toward the development of a heroin vaccine will give us new tools to prevent and treat heroin addiction.

- 3) *Prevention, Education, and Outreach.* NIDA is also committed to translating and disseminating the products of the research we support into practice for use by the public and public health professionals. Education is a critical component of our efforts to curb the abuse of prescription medications and must target every segment of society, including doctors. For over 10 years NIDA has been using a multi-pronged approach to advancing addiction awareness, prevention, and treatment in primary care practices, including the diagnosis of prescription drug abuse. This effort included supporting several Centers of Excellence for Physician Information, located at some of the most prestigious medical schools in the country. By targeting physicians-in-training, curricula developed are used in classrooms across the country to help prepare the next generation of physicians to intervene with patients early in their care.

NIDA will also continue its close collaborations with the Office of National Drug Control Policy (ONDCP), SAMHSA, and other Federal agencies, as well as professional associations with a strong interest in preserving public health. NIDA recently sponsored a 2-day meeting in conjunction with the American Medical Association and NIH Pain Consortium, where more than 500 medical professionals, scientific researchers, and interested members of the public had a chance to dialogue about the problems of prescription opioid abuse and to learn about new areas of research.

2. There is also a dangerous trend developing amongst opioid abusers of turning to heroin because it is cheaper and in some cases more readily available from dealers than black market prescription drugs. However, intravenous heroin use brings with it a slew of other problems that tax our public health system, such as hepatitis C, HIV, bacterial infections, and more. Can you tell us how NIH research on substance abuse is going to benefit public health as a whole?

Answer:

Recognizing the dangerous trends and consequences associated with the diversion, non-medical use, and abuse of opioids, NIDA continues to seek and support additional research on the subject and to inform the public, physicians, pharmacists, and others. NIDA first launched its prescription drug abuse public health initiative in 2001. Through NIDA's support of surveillance mechanisms, including our Community Epidemiology Work Group and the Monitoring the Future survey, we continuously monitor trends in all forms of drug abuse, including the abuse of prescribed medications and illegal opioids like heroin.

On the acute side of the equation, NIDA has developed a robust research portfolio related to preventing overdoses. Making the effective opioid-overdose antidote *naloxone* more available and user friendly will help prevent many deaths. The FDA recently approved a hand-held auto-injector of naloxone that patients and others can use. NIDA is also supporting the development of a naloxone *nasal spray* to be used by non-medical personnel including the patient. Also, since many overdoses occur when no one is around, or during

sleep, NIDA is supporting the development of self-activated systems that initiate an emergency response when wireless sensors signal that an overdose is occurring.

Another critical piece of the opioid puzzle to come out of NIDA sponsored research in this area pertains to the transformative public health potential of more broadly integrating drug treatment into healthcare settings. Research clearly shows that medication-assisted treatment will be most effective when offered within the larger context of a high-quality delivery system that address opioid addiction not only with medication but also with behavioral interventions to support treatment participation and progress, infectious disease identification and treatment (especially HIV and HCV), screening and treatment of comorbid psychiatric diseases, and overdose protection (naloxone). NIDA's research over the last 2 decades has provided us with evidence that a high quality treatment system to address opioid addiction must include all these components, yet there are currently very few systems in the U.S. that provide this bundle of effective services. Healthcare reform—with a focus on both expanding access to treatment and improving the quality of care—offers hope that we may be better able to integrate drug treatment into healthcare settings and offer comprehensive treatment services for opioid addiction. We are also examining ways to use healthcare reform and the focus on health promotion and wellness to pay for and deliver prevention interventions targeted at children, adolescents, young adults, and high-risk adult populations like those with chronic pain or returning veterans.

Finally, NIDA partners with other federal agencies (CDC, SAMHSA, ONDCP and ONC), in implementing and evaluating evidence based interventions towards controlling the epidemic of prescription overdose deaths in our country.

Asthma/Allergy Research

Question for Dr. Collins

Question:

Dr. Collins, in November Senator Shaheen and I wrote to Secretary Sebelius to express our support for the Guidelines for the Diagnosis and Management of Asthma. Given the important role that allergies play with asthma attacks, visits to the emergency room and missed schools days, I remain concerned that we are proceeding too slowly with regard to the guidelines. Dr. Gibbons who leads the National Heart, Lung, and Blood Institute which oversees the asthma guidelines responded to our letter. His response affirmed the NIH commitment to the guidelines and outlined steps toward implementation. I would like a written response as to where matters stand with the following milestones outlined in the report.

1. Convene a national policy forum to include entities such as commercial and public health plans, professional associations, experts in performance measurement, public and private healthcare financing organizations, patient advocacy groups, employers, workplace advocacy groups, state and local policymakers, environmental, school, and other national, state, and local agencies.

2. Strategy 3: Convene a workgroup of primary care providers, allergists, representatives of health plans, and state Medicaid Medical Directors to explore barriers to allergy testing in primary care settings to reach consensus on, and implement policies for, supporting the use of allergy testing in accordance with EPR-3 recommendations; and, to facilitate referrals to specialists, as appropriate, for consultation or co management of patients.
3. Strategy 1: Convene managed care companies to work with NCQA to develop a HEDIS measure of environmental assessment and monitoring (including monitoring success with adherence to an allergen/irritant exposure control strategy); prepare a dissemination and implementation plan for the HEDIS measure and tools; garner broad support for use of the measure and accompanying implementation tools.

Answer:

The National Asthma Education and Prevention Program's (NAEPP) Guidelines Implementation Panel Report offers suggested strategies to enhance dissemination and adoption of key recommendations in the Guidelines for the Diagnosis and Management of Asthma. Rather than designating the strategies as items for the NAEPP to accomplish on its own, the strategies were offered as a list of possible activities for NAEPP member organizations and other professional, private sector, state and local government, and patient groups to consider undertaking within their respective organizations in order to improve asthma care.

Many organizations have done so. For example, The American Lung Association, The Public Health Foundation, and the MERCK Childhood Asthma Network (MCAN) at the George Washington University School of Public Health and Health Services have all convened national groups and prepared reports that address a range of policy issues (including enhancing control of environmental asthma triggers) and suggest policy reforms and other actions for policymakers, health care administrators, and a wide variety of stakeholders involved in health care to consider. The Allergy and Asthma Network/Mothers of Asthmatics, a patient stakeholder group, holds annual meetings on Capitol Hill to update legislators on critical issues facing patients and their families who have allergies and/or asthma.

Working groups at the local level have made considerable progress in engaging primary care providers, allergists, and representatives of health plans, including Medicaid, to identify and overcome local barriers and accelerate implementation of recommendations in the guidelines, including those relating to control of allergens. For example, the Center for Medicaid Services Health Care Innovation Awards Program included 5 awardees that address asthma and all of these programs incorporate attention to environmental allergens. The Environmental Protection Agency's vibrant Community Network and annual EPA leadership Awards program offer outstanding examples across the country of community organizations, clinicians and health care administrators, including Medicaid service providers, working together on programs that focus on incorporating measures to control

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environmental asthma triggers, including allergens, into comprehensive asthma management. The Centers for Disease Control and Prevention's National Asthma Program and NHLBI's National Asthma Control Initiative showcase tools and programs (e.g., home visit guides, continuing education programs for primary care, and patient materials) developed by state public health and local community clinics that can be adapted by other stakeholders

As is illustrated above, National professional societies and patient groups and local healthcare and community groups have and will continue to make considerable progress in engaging primary care providers, allergists, and representatives of health plans to identify and overcome local barriers and accelerate implementation of recommendations in the Guidelines.

Cancer Research Funding Disparity

Earlier this month, the American Society of Clinical Oncology in its State of Cancer Care in America report announced that by 2030 cancer will become the leading cause of death in the United States. Yet over nine of the last ten years, including in the Administration's FY15 proposed budget, the National Cancer Institute (NCI) received a diminishing share of the NIH's budget.

Given this trend, do you think we are investing enough in cancer research?

Answer:

NCI has received a slightly lower portion of the overall NIH budget in recent years because some NIH funds have been assigned to trans-NIH efforts like the Brain Research, through Advancing Innovative Neurotechnologies (BRAIN) Initiative, and Alzheimer's disease research. Also, in the years immediately after the September 11, 2001 attacks on the U.S., a new research priority arose to address the needs to improve the availability of medical countermeasures against potential biological, nuclear, radiological, and chemical threats, which had a significant impact on the relative allocation of resources between the ICs. Furthermore, while NCI is responsible for more than 80 percent of the NIH cancer research investment, there are multiple ICs working on cancer research, either independently or in collaboration with the NCI.

Submitted by Rep. Lucille Roybal-Allard **Questions for the Record for NCI Director Dr. Harold Varmus**

Status update on pancreatic cancer research

During the hearing, we ran out of time while you were answering my first question about the status of pancreatic cancer research at the NCI.

Pancreatic Cancer Research

Question 1:

We quickly spoke about potential for research in the area of pancreatic cancer and diabetes, but we ran out of time. So I would like to re-ask the question: will the NCI be issuing a Funding Opportunity Announcement (FOA) in this area, and if so, when will the announcement be made and how much funding will be devoted to it?

Answer:

The NCI's Scientific Program Leadership (SPL) group recently considered a proposal for a new Trans-NIH consortium composed of NCI, NIDDK, and NIAAA to support research on relationships between chronic pancreatitis, diabetes, and pancreatic cancer, as well as biomarkers and methods for early detection of pancreatic cancer. The consortium members are seeking approval from their respective national advisory boards to issue a Request for Applications (RFA). The NCI SPL approved the concept for presentation to NCI's Board of Scientific Advisors in June 2014. The RFA will involve all the scientific divisions of the NCI, as well as NIDDK and NIAAA, and will require extensive planning and coordination. If approved, the RFA is expected to be issued by the end of the year, with an anticipated funding level of approximately \$30 million over 5 years, with \$10 million from NCI. It is important to note that these are estimates; actual funding levels will be contingent upon the level of appropriations.

Question 2:

I understand that immunotherapy has great potential to be effective against some cancers, and without the devastating side-effects of chemotherapy. In the next year, what specific steps and programs is NCI exploring to address immunotherapy in pancreatic cancer treatments, and do you have a specific funding amount in mind for these activities?

Answer:

There has been remarkable progress in the development of several kinds of cancer immunotherapies over the past few years, based on decades of basic research on the immune system. Despite the exciting successes, however, not all cancer types have been responsive, not all patients with the most promising cancer targets have responded, and significant side effects have been observed in many cases. Although some tumors, generally referred to as "solid tumors" (as opposed to blood cancers), have been more difficult to approach with immunotherapies, there have been successes in both patients and animal models of cancer, and some of these successes have occurred with pancreatic cancers.

The report recently delivered to Congress in response to the Recalcitrant Cancers Act specifically describes plans to build upon recent advances in immunotherapy to improve our ability to treat pancreatic cancer. Further progress in developing pancreatic cancer immunotherapies will require the discovery and validation of new immunotherapy targets, the rational combination of immune modifiers and both conventional and targeted drugs in preclinical and clinical studies, and the production of immune-modulatory molecules (such as anti-CD40). We expect much of this work to be performed through grants to individual investigators and teams, some at the NCI's Frederick National Laboratory for Cancer

Research (FNLCR), and some through the Cancer Immunotherapy Trials Network (CITN), which employs the collective expertise of expert academic immunologists. We will also work with foundation and industrial partners to design and conduct cancer therapy trials with the most promising immunotherapy agents against several cancer types, including pancreatic ductal adenocarcinoma (PDAC).

As is always the case, NCI's support of such work will be contingent upon our appropriated budget and the submission of meritorious proposals. The amounts provided for each project will differ, reflecting the actual needs of the proposed projects. In addition, NCI will continue to support research on a variety of approaches to immunotherapy, including research focused on immune response modifiers, identification of tumor antigens, and the development and use of therapeutic vaccines in combination with other agents, in addition to our traditional and essential support of the fundamental immunology that has made recent therapeutic advances possible.

Question 3:

One of the research recommendations is to focus on developing biomarkers for the early detection of pancreatic cancer. The value of early detection would be to prevent further development of the disease. So, are there any potential vaccines, or treatments, in the pipeline to prevent the development of pancreatic cancer?

Answer:

There is consensus that the discovery of biomarkers that can identify early lesions and perhaps serve as therapeutic targets is a critical goal in advancing progress against PDAC; the diagnosis of pre-invasive or even small cancers can improve resectability, the prognosis after resection, and survival. To date, there are no biomarkers or panels of biomarkers that are sensitive and specific enough to allow routine use in the diagnosis of PDAC in its early stages. The NCI has been investing in the development of early detection biomarkers for many kinds of cancer through its Early Detection Research Network (EDRN). As described in the NCI's recent report to Congress on PDAC, we are taking advantage of new knowledge about genetic and other risk factors (pancreatic cysts and newly diagnosed diabetes mellitus [see Q1/A1]) to test novel approaches to early diagnosis. The NCI plans to develop a Program Announcement that focuses on these issues in the near future.

Submitted by Rep. Lucille Roybal-Allard
Questions for the Record for NIH Director Dr. Francis Collins

Health economics research at NIH

Thank you for answering many of the questions my colleagues and I had regarding the status and future of health economics research at the NIH. We did have one question that went unanswered and we wanted to ask it for the record.

Questions:

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1. We have heard that fewer Institutes are participating in OppNet, the only trans-NIH initiative focused on basic behavioral and social science research. Is this correct?

Answer:

No, this is not the case. Between October 2010 and September 2013, the Basic Behavioral and Social Science Opportunity Network (OppNet) provided \$64 million to fund 150 extramural research projects supported by 24 NIH Institutes and Centers (ICs) and four Program Offices within the Office of the Director. IC participation has remained constant since the creation of the program. OppNet has generated so many innovative applications that various ICs have funded all or part of 23 projects in addition to those supported by the OppNet budget.

Further,

2. Can you give the Committee a sense what the historic participation level in OppNet has been?

Answer:

Twenty-four NIH Institutes and Centers and four Program Offices within the Office of the Director have funded and managed OppNet annually since the inception of the program in Fiscal Year (FY) 2010 to the present. OppNet's budget began in FYs 2010 and 2011 at \$12 million per year. The annual budget grew to \$22 million in FY 2012 and \$20 million in FY 2013.

3. What is the current participation level at this point?

Answer:

As they have done for each year since OppNet was established, 24 NIH Institutes and Centers and four Program Offices within the Office of the Director participate in the OppNet program. We estimate the budget for FY 2014 to be approximately \$20 million.

Congresswoman Barbara Lee – Questions for the Record

Neuromyelitis Optica

Question:

Neuromyelitis Optica (NMO) is a rare disease with limited research mostly funded by the NINDS. The NIAID, on the other hand, funds genetic susceptibility research in rheumatologic diseases.

1. How does the NIH encourage collaboration among scientists and NIH Institutes to find common genetic variants that pre-dispose to a wide variety of autoimmune diseases?

Answer:

The National Institute of Allergy and Infectious Diseases (NIAID) is the leading institute at the National Institutes of Health (NIH) for research on immunologic disorders, including autoimmune diseases. The National Institute on Neurological Disorders and Stroke (NINDS) also funds research on autoimmune diseases that affect the nervous system, including NMO. Collaboration and coordination is facilitated through regular meetings held by formal committees and working groups as well as collaborative research centers. NIAID chairs and NINDS participates on the NIH Autoimmune Diseases Coordinating Committee (ADCC) that facilitates coordination of research across NIH, federal agencies, professional societies, and patient and advocacy organizations with an interest in autoimmune diseases. NIAID funds the Autoimmunity Centers of Excellence that conduct collaborative research on autoimmune diseases and support partnerships among clinicians and basic researchers to accelerate the translation of scientific advances to the clinic. NIAID also supports the Cooperative Study Group for Autoimmune Disease Prevention (CSGADP) to engage in scientific discovery that significantly advances knowledge for the prevention and regulation of autoimmune disease. A major goal of the CSGADP is to advance understanding of the genetic and environmental risks for autoimmune disease.

2. How can we bring everyone to the table so they can learn from each other?

Answer:

NIH brings together key stakeholders and encourages research coordination across scientific fields, including among NIH Institutes and Centers. As part of this effort, NIH organized the Center for Human Immunology, Autoimmunity, and Inflammation (CHI), a cooperative, trans-NIH research initiative, to efficiently translate enhanced understanding of immune function and pathophysiology to the clinic. Ten NIH Institutes participate in the CHI, and a number of clinical studies are underway. CHI research focuses on human immunology, with an emphasis on shared mechanisms that underlie immune disease, including autoimmunity. CHI provides high-throughput technologies for large scale examination of the genomes of volunteers with various autoimmune diseases. Information derived from the CHI will be helpful to NIH researchers, as well as extramural researchers, in the field of autoimmune diseases research.

3. Is anyone at NIH actively seeking potential collaborations to bring scientists together on these issues?

Answer:

To continue to encourage research collaborations on autoimmune diseases, the NIAID has re-competed its Autoimmunity Centers of Excellence (ACE) in fiscal year 2014. Established in 1999, ACE basic and clinical researchers work together to design, fully

develop, and conduct clinical trials and integrated mechanistic studies of immune-modulating agents for the treatment of autoimmune diseases. The findings of this collaborative research effort are helping to increase understanding of autoimmune disease and accelerate scientific advances to patients. In addition, the NIH invites all interested NIH intramural researchers to join the Center for Human Immunology, Autoimmunity, and Inflammation (CHI) and work toward the goal of cooperative research to understand human immunology, including autoimmunity. CHI hosts meetings, lectures, and conferences for researchers to discuss new developments in the field of immunology.

4. I understand that "Personalized medicine" has become a very popular topic -- matching therapies to individuals based on their unique genetics. Is there a plan at the NIH to provide whole genome sequencing for rare diseases like NMO?

Answer:

NIH is exploring the use of whole genome sequencing and another genome analysis method called whole exome sequencing (sequencing of the genes only and not the rest of the genome) in the diagnosis of rare diseases through a number of different research programs. One example is the Undiagnosed Diseases Program (UDP), which is a trans-NIH intramural initiative led by the National Human Genome Research Institute (NHGRI) that was launched as a pilot program in 2008. The UDP has two goals — to provide answers to patients with rare, mysterious conditions that have long eluded diagnosis, and to advance medical knowledge about human diseases. Patients are referred to the UDP by their clinicians and, if accepted, travel to the NIH Clinical Center in Bethesda for intensive weeklong evaluations, including the collection of samples for DNA sequencing. Currently the UDP team reviews approximately 60 completed applications each month and accepts approximately 130 patients each year.

Such has been the success of the UDP that NIH is now establishing a nationwide program to build on the UDP called the Undiagnosed Diseases Network (UDN). Funded by the Common Fund, the UDN will increase NIH's capacity to conduct research on undiagnosed diseases through funding a network of multi-disciplinary centers across the country. To date, an award has been made to Harvard Medical School for a Coordinating Center for the UDN, and this summer NIH will announce five to seven UDN clinical sites that will form the Network. The Network will provide greater access to patients from diverse geographic populations and enable the development of tools for use in many different hospital settings, which can then be shared beyond the UDN.

NHGRI is further utilizing DNA sequencing technology to understand the genetic causes of rare diseases through the Centers for Mendelian Genomics (CMGs). We have made remarkable progress since the beginning of the Human Genome Project in 1990. Since that time, the number of diseases where we understand the molecular basis has increased from around 61 to over 5,000. Nevertheless, there remain many rare diseases that are believed to be caused by DNA changes in single genes in the genome — 'Mendelian disorders' — but where the gene affected has not been identified. The main goal therefore of the CMGs is to employ sequencing across the genome to discover the genetic changes causing these Mendelian disorders. Over the past two years, the CMGs have identified genetic changes

in over 200 genes that cause diseases. Knowing what causes a disorder will increase our understanding of its biology, and may improve diagnosis and treatment options.

Lupus

Question:

Lupus is a very complex, autoimmune disease impacting each organ system and person differently. Research in lupus has come a long way, but there is still so much work to be done. People with lupus need and deserve an arsenal of safe and effective treatments.

The AMP program is a positive step forward in recognizing the need for more research in a disease like lupus. But there is significant concern within the lupus research community that these are not new dollars allocated to lupus, and lupus research funding is already significantly lagging. As of March 7, NIH states through its online system (RePORTer) that \$92 million was allocated towards lupus research in FY 13 versus \$108 million in FY 12. A \$16 million decrease over one year in vital funds for a complex and drastically underfunded disease like lupus is disconcerting.

Lupus scientists are struggling to stay afloat, and labs are closing all over the country. We are losing committed and passionate researchers for a disease in which there is already a dearth of young investigators.

1. Imagine you are a person living with lupus and you hear this great announcement of a major NIH initiative that actually includes lupus. However, you also see a \$16 million decrease in lupus research funding from FY 12 to FY 13. With the AMP announcement, you would logically think the lupus budget would increase and not decrease especially at a time in which more research dollars are desperately needed. It is a mixed and confusing message for people with lupus and the research community. How do you explain this?

Answer:

Let me assure you, the NIH has a long history of supporting lupus research and currently funds a broad portfolio of studies to better understand the multiple aspects of lupus, across the spectrum of basic, translational, and clinical research. A number of NIH Institutes and Centers, including the National Institute of Arthritis and Musculoskeletal and Skin Diseases, the National Institute of Allergy and Infectious Diseases, the National Heart, Lung, and Blood Institute, the National Institute of Diabetes and Digestive and Kidney Diseases, the National Institute of Environmental Health Sciences, the National Institute of Neurological Disorders and Stroke, the *Eunice Kennedy Shriver* National Institute of Child

Health and Human Development, and the National Institute on Minority Health and Health Disparities support research on various aspects of the disease.

In FY 2014, NIH launched the AMP initiative and we are pleased to be partnering with the U.S. Food and Drug Administration, industry and non-profit organizations in the Accelerating Medicines Partnership (AMP) to speed the development of new therapeutics for lupus, rheumatoid arthritis, type 2 diabetes, and Alzheimer's disease, through research focused on identifying and validating biological targets for new therapeutics, a process called target validation. Over half of drugs fail in phase II and phase III clinical trials due to lack of efficacy, and improvements in the target validation process should reduce that failure rate. Funding for the AMP program in lupus and rheumatoid arthritis is not expected to begin until late fiscal year (FY) 2014. Therefore, the FY 2013 NIH funding numbers for lupus do not include AMP funding. Because the overall NIH budget decreased between FY 2012 and FY 2013, funding levels for research in many disease areas also decreased during this time period. When considering the categorical NIH funding levels, it is important to recognize that scientific breakthroughs are often serendipitous; NIH-funded research, particularly basic research that is not necessarily categorized as lupus-related, may yield important insights that advance understanding and treatment of the disease.

2. With a \$16 million decrease in lupus funding from FY 12 to FY 13, how does the NIH justify taking vital research dollars away from an already hit and struggling lupus field for AMP? What is your thinking in terms of redirecting funding from other vital lupus research?

Answer:

Overall NIH dollars declined from \$108 million in FY 2012 to \$92 million in FY 2013, due in large part to the sequester. For the most part, the NIH does not assign funding based on set-aside allocations for specific diseases. The National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), the NIH Institute with primary responsibility for lupus research, invests the majority of its extramural budget in the most outstanding investigator-initiated research—research for which an individual investigator, rather than the NIAMS, chooses the disease area or topic of study. The Institute's funding priorities reflect highly meritorious research as determined by factors such as peer review by experts in the field, public health needs, and scientific opportunities. A small proportion of NIH-sponsored research is funded in response to initiatives developed by the NIH Institutes and Centers to pursue specific scientific opportunities or to address unmet needs. NIH initiatives are publicized to the research community through Requests for Applications (RFAs) and Program Announcements—public solicitations for grant applications to address a specific research area.

The Accelerating Medicines Partnership (AMP) addresses the high priority need to transform the current model for developing new diagnostics and treatments, through better

target validation at the outset aimed at reducing the current failure rates in late stage clinical trials. The program also offers an exceptional opportunity to leverage NIH investments in lupus research with substantial funds and intellectual support from industry and non-profit organizations. Recognizing the need and opportunity, the NIH, after consulting with the research community, released two RFAs to implement the AMP program in lupus and rheumatoid arthritis. The RFAs will not take money away from existing lupus projects. We expect that a number of researchers studying lupus will apply and be funded through the AMP. Because a major goal of the AMP is to generate pre-competitive, disease-specific data that will be accessible to the broad biomedical community, the program will also facilitate research by lupus investigators not funded through the AMP.

Health Disparities and College-aged African-American Women

Question:

Dr. Beverly Daniel Tatum, President of Spelman College, testified before this subcommittee during the Public and Outside Witnesses Hearing on behalf of UNCF (the United Negro College Fund).

During a conversation with her following the hearing, she informed me that Spelman has been engaged in conversations with NIH (through the National Institute on Child Health and Human Development (NICHD)) related to health disparities that affect college-aged African American women.

In this regard, Spelman has launched a wellness initiative that promotes healthy lifestyle choices that students can maintain into adulthood.

1. What research and prevention efforts are underway at NIH that engage African American college-aged women on wellness and disease prevention?

Answer:

NIMHD is testing an intervention to improve health for African American women aged 18-25 through an innovative internet-based health communication system that provides personalized health information through interaction with an animated character.

Preliminary results indicate that women enjoy interacting with the virtual character, find the health information provided to be useful, and feel more confident after the interaction talking to their doctor about health issues. Another NIMHD-funded study uses data from the National Longitudinal Survey of Youth to examine the role of pregnancy and child-rearing on obesity across the life course for African American, white, and Latina women. Investigators have found that childbearing in young adulthood was associated with obesity 25 years later only among African American women with multiple pregnancies who were overweight at the time of childbearing. Findings suggest that targeted obesity interventions may be needed for this subgroup of women at elevated risk for permanent weight gain.

NIDDK's Weight-control Information Network (WIN) coordinates the *Sisters Together: Move More, Eat Better* program (<http://www.win.niddk.nih.gov/sisters/index.htm>), which is designed to encourage African American women ages 18 and older to improve their health by making healthier food choices and getting regular physical activity. The *Sisters Together* series consists of science-based and culturally appropriate brochures and program guides that address the specific barriers that black women may have to exercising and eating better. The NIDDK's Short-Term Research Experience Program for Underrepresented Persons (STEP-UP) and Diversity Summer Research Training Program (DSRTP) provide summer research training opportunities for college students from groups underrepresented in biomedical research, including African American women.

NHLBI funds research investigating the best ways to prevent weight gain in minority women during adulthood. One project builds upon recent work which showed that an innovative intervention based on self-affirmation was successful in motivating behavior change in obese African American and Latino adults, specifically increasing physical activity in overweight/obese minority women and men with coronary artery disease. Several NIH Institutes and Centers support research efforts addressing the health and prevention of disease in African American college-aged or young adult women. For example, NICHD recently funded a career development grant to use a community-based participatory research approach to identify the barriers and facilitators to offering HIV testing at a Historically Black College/University. This research engaged both female and male African American students in developing non-traditional settings for this critical public health testing.

2. Are there any follow-on programs or initiatives that NICHD has proposed in its work with Spelman College?

Answer:

Arising from Spelman College's Wellness Revolution, NICHD and Spelman College entered into a partnership in early 2014 to cooperate on projects to prevent disease and disability and promote the health of Spelman students, faculty, and staff. These projects will include nutrition, fitness education, and physical activity promotion. In February 2014, NICHD and Spelman cosponsored a wellness summit at Spelman, which brought Institute and college leadership together with experts on nutrition in women's health and physical activity, including the President's Council on Fitness.

NICHD and Spelman will also connect students to health researchers through internships and other educational opportunities to foster interest in scientific careers. Another goal of the partnership is to apprise potential national partners, including other NIH Institutes and Centers, about the Wellness Revolution as a possible model program.

Sickle Cell

Question:

Please describe the current research being done to further identify the health risks associated with sickle cell trait, and to proactively identify this trait in at-risk populations who were not tested for the trait at birth.

Answer:

Sickle cell disease (SCD) is a genetic blood disorder, in which the body produces abnormal hemoglobin resulting in sickle-shaped red blood cells that can block blood flow in the limbs and organs causing pain and organ damage. People who have two copies of the sickle hemoglobin gene have SCD. However, people who inherit a sickle hemoglobin gene from one parent and a normal gene from the other parent have sickle cell trait (SCT) and they produce both normal and sickle hemoglobin. SCT has been demonstrated to afford a survival advantage in regions where malaria is widespread and individuals with SCT usually have few, if any, symptoms and lead normal lives. However, SCT individuals can develop medical complications, including kidney failure and malignancy, blood clotting disorders, and exercise-related sudden death.

The National Heart, Lung, and Blood Institute (NHLBI) has supported research that has and continues to improve the lives of patients with SCD. As recently as 1970, the average patient with SCD died in childhood, but patients with SCD now can live to the mid-50s to 60s. While SCD has no cure, the results of NHLBI-funded clinical trials have led to the use of penicillin to prevent fatal infections, chronic blood transfusion to reduce stroke risk, and hydroxyurea to reduce pain, dramatically improving the lifespan of children with the disease. Current research efforts promise to yield continued improvements in longevity and quality of life. Particularly exciting are studies that attempt to raise the level of fetal hemoglobin (not affected by the sickle cell gene and a powerful modifier of the severity of SCD), through modulation of a gene called Bcl11A.

Less well understood, though, are the adverse health effects of SCT, due in part to difficulties in conducting large scale epidemiological studies in which data on SCT and associated clinical outcomes are available. For this reason, the NHLBI is collaborating with the Uniformed Services University of the Health Sciences on a project, "Determining Health Effects Attributable to HbAS (Sickle Cell Trait) Through Analysis of US Military Data" that will mine existing military databases that contain decades of health information to help us better understand the health risks associated with sickle cell trait.

Whether an individual with SCT develops symptoms or not, they can pass the gene to their children, so accurate diagnosis and counseling are important for those who want to be informed of their status. All U.S. states and Washington, DC mandate testing for SCD as part of their newborn screening programs. The test can also show whether a newborn infant has SCT, i.e., sickle hemoglobin in addition to normal hemoglobins, however, standardized communication of this information to the infant's parents remains a challenge to be addressed. NHLBI will also support research to develop low cost devices to diagnose both SCD and SCT. These low cost devices will be useful for proactive onsite diagnosis of SCT in both low and high resource settings using very small amounts of blood, in order to promote broader testing of SCT.

Lung Cancer

With regard to the Recalcitrant Cancer Research Act, I understand that in preparing the Scientific Framework for lung cancer, NCI has chosen to focus only on small cell lung cancer, which represents 10-15% of lung cancer deaths, while leaving out non-small cell lung cancer, which represents the vast majority of lung cancer deaths, and includes adenocarcinoma, the most common type of lung cancer in non-smokers. Congress's expectation is to receive a Scientific Framework from NCI that addresses all forms of lung cancer - both small cell and non-small cell.

Question: Will NCI meet this expectation in July and deliver to Congress a comprehensive Scientific Framework that includes all types of lung cancer? If not, why not?

Answer: The NCI is fully committed to meeting the specific requirements of the RCRA and has begun with two important types of cancer that meet the criteria stated in the legislation. Thus a "scientific framework" report on pancreatic ductal adenocarcinoma (PDAC) has already been delivered to Congress and a report on small cell lung cancer is nearing completion.

The difference between your expectation of a report on "lung cancer" in all of its forms and the NCI's choice of small cell lung cancer (SCLC) as a second type of cancer to be subject to the RCRA reflects an important change in the classification of cancers. As our knowledge about cancer biology has increased over the last several years, we have come to understand that the classification of cancers used in the past - grouping all cancers that arise in one organ, such as the lung or the pancreas, as one kind of cancer, with multiple subtypes - is no longer appropriate.

Of the three relatively common forms of cancer found in the lung, small cell lung cancer (SCLC) arises from neuro-endocrine cells, squamous cell lung cancers from the squamous epithelium in the large, central tubes in the bronchial system, and adenocarcinoma of the lung from the pneumocytes in the lung periphery. We recognize these as three different kinds of cancer arising from three different cell lineages, each tumor type exhibiting mostly different mutational profiles (as documented by recent NCI-funded genomic studies), with different clinical histories, different treatment options, and even different investigators working on these three different types of cancer. In addition, the amount of research progress has varied for these three cancers arising from the three lung cell lineages (neuro-endocrine, squamous epithelium, and adenocarcinomas). As a consequence, each of the three major types of cancer that originate in the lung present very different problems, requiring different solutions, as revealed at the workshop described below. Similarly, we reported on only one of the two kinds of cancer arising in the pancreas - pancreatic ductal adenocarcinoma (PDAC), but not islet cell carcinomas - in part because the diseases arise in different cell lineages and present different problems for investigation and for cancer control (prevention and treatment).

To produce our forthcoming “framework” report on SCLC, NCI followed the same approach used in developing the framework for PDAC. We began by planning a “horizon scanning” workshop. Goals of both workshops were to identify new ideas and important, unsolved problems in relevant fields of investigation and to identify approaches to solve those problems. As the forthcoming report will describe, treatment of SCLC has not changed in the last 30 years, avoidance of the use of tobacco is the only known way to prevent the disease, no screening method has proved effective, responses to chemotherapy fluctuate and are difficult to understand, and life expectancy after diagnosis tends to be very short. In many ways, these features differ from those observed with the other common forms of lung cancer, although those too have outcomes that need to be greatly improved. In addition, there are very limited materials available for the study of SCLC; the report will describe a number of ways in which the NCI hopes to remediate this situation, in part by developing new research materials and in part by stimulating interest among investigators to propose new research on SCLC, thereby increasing the number of active research projects that address SCLC.

The SCLC workshop was held in July, 2013. Since then NCI has been following up on the findings and developing the framework report; we anticipate meeting the July deadline to send our report to Congress. Although we have no immediate plans to produce a framework for other kinds of cancer (arising in the lung or elsewhere), we continue to support a great deal of research on lung adenocarcinoma and squamous cell carcinoma. It is also important to note that the NCI does not confine its evaluations of research on certain cancer types to those conducted under the RCRA. Meetings of NCI and extramural experts to conduct “horizon scanning” for scientific opportunities on a variety of cancers occur as part of NCI’s standard practices, as recently done, for example, with hepatic and gastric cancers. We will, of course, continue to comply with other provisions of the RCRA over the coming years.

Multiple Sclerosis/BRAIN Initiative

Multiple Sclerosis/BRAIN Initiative

Question:

1. How has the recent development of specialized projects, such as the BRAIN initiative, impacted the NIHs ability overall funding ability outside the projects' scope?

Answer:

NIH’s funding decisions balance scientific opportunity, scientific merit and technical excellence, public health need, and portfolio diversity. Careful resource allocation ensures that NIH can strategically address the public health challenges of today and be poised to move quickly when new public health threats or unexpected scientific opportunities emerge, like the BRAIN Initiative. The BRAIN Initiative represents an exciting opportunity to build on recent success in neuroscience research and to focus investments from NIH and other funders to make significant progress in understanding the brain’s functions and its complex links to behavior and disease. Support of the BRAIN Initiative will undoubtedly have far-reaching impacts beyond the scope of the project itself as

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scientists understand more about the causes of diseases and disorders linked to the brain and develop ways to treat and cure them. It is important for NIH and its Institutes and Centers (ICs) to retain budget flexibility to support its mission. This flexibility enables NIH to pursue multiple avenues of research simultaneously and to develop strategies for effective investment across all areas of scientific research that are necessary for improving human health.

2. What is being done with the BRAIN initiative to directly engage the patient community?

Answer:

While the immediate focus of the BRAIN Initiative is fundamentally basic science, the NIH remains cognizant of the importance of consulting with the patient community. The perspectives of patients and their advocates are critical to achieving the ultimate goal of the BRAIN Initiative, which is to improve our abilities to diagnose, prevent, and cure debilitating brain diseases and disorders. For this reason, the BRAIN working group charged with developing the scientific plan for the Initiative has provided multiple opportunities for public input at the majority of its meetings. This input included comments from patient advocacy groups, physicians, and members of the lay public. The NIH has also been working with numerous patient advocacy organizations to maintain close communication, and, in 2013, NIH and the American Brain Coalition (a non-profit organization comprising over 60 of the United States' leading professional neurological, psychological, and psychiatric associations and patient organizations) hosted a webinar to engage patients and their advocates in a discussion about the BRAIN Initiative. As the Initiative matures, NIH will continue to engage patients and the public to ensure that efforts are beneficial for all stakeholders.

General Research Funding Question

Answer:

The Small Business Innovation Research (SBIR) and Small Business Technology Transfer (STTR) programs are critical in leveraging the NIH investment in life science small businesses and helping the agency to accomplish its mission to improve public health. The main goals of the SBIR/STTR programs are to increase innovation and participation among small businesses to meet federal R&D needs, while creating future opportunities for possible commercialization of these efforts. Both programs are a three phase activity in which grants are intended to fund small businesses with the initial startup costs or seed money needed to fund early stage R&D. The three phases include Phase I to establish technical merit and feasibility, Phase II to continue R&D, and Phase III to advance toward commercialization through non-SBIR/STTR third party investments and partnerships. The STTR program differs from the SBIR program, because it requires the small business concern to partner with a non-profit research organization.

NIH funded over 800 competing SBIR/STTR awards, over 600 continuing projects, and over 50 supplements to ongoing projects in FY 2013; these awards went to approximately 1,100 small business concerns. Roughly 1/3 of NIH SBIR/STTR awardees are first

time applicants and award winners. The ‘typical’ NIH small business awardee is quite small, usually from 3-20 employees.

NIH conducts extensive outreach to small businesses across the country. The purpose of this outreach is to educate the small business community about the programs and to encourage their participation. NIH’s SBIR/STTR outreach activities during FYs 2012-2013 were directed at identifying new SBIR/STTR applicants, with a special emphasis on women-owned businesses, socially and economically disadvantaged businesses, and under-represented states (IDeA states). NIH conducted targeted outreach to these under-represented groups through collaboration with SBA and partnering SBIR and STTR governmental agencies, including NSF, DOD, DOE and NASA. NIH participated in webinars, conferences, and in-person events throughout the country as part of our outreach effort.

NIH continues to make a special effort to stimulate research at educational institutions, which have not been major recipients of NIH funds, but provide baccalaureate and/or advanced degrees to many of the Nation’s scientists. This initiative is the Academic Research Enhancement Award (AREA) program, which enables qualified scientists to receive funds for small-scale projects.

AREA grants aim to create research opportunities for scientists who are otherwise unlikely to participate in NIH biomedical research programs. Such opportunities create benefits for scientists and their institutions as it is anticipated that investigators funded by AREA benefit from the opportunity to conduct an independent research project; the grantee institution benefits from a research environment that is strengthened by the AREA program and enhanced through participation in the diverse extramural programs at NIH; and the students at recipient institutions benefit from exposure to and participation in biomedical research.

In addition to the SBIR/STTR and AREA programs discussed above, NIH continues to encourage any institution to apply for other research program grants such as the R01.

IC Director's Office Cost Summary
(Amounts in Dollars)

| IC by Year | FY 2011 | FY 2012 | FY 2013 | FY 2014 (est.) | FY 2015 (est.) |
|-------------------|----------------------|----------------------|----------------------|-----------------------|-----------------------|
| NCI..... | \$ 5,038,041 | \$ 4,301,151 | \$ 3,465,661 | \$ 3,494,816 | \$ 3,508,594 |
| NHLBI..... | \$ 1,544,680 | \$ 1,282,867 | \$ 1,687,648 | \$ 1,703,954 | \$ 1,720,423 |
| NIDCR..... | \$ 821,551 | \$ 1,056,990 | \$ 1,051,350 | \$ 1,249,447 | \$ 1,404,300 |
| NIDDK..... | \$ 2,780,276 | \$ 2,495,358 | \$ 2,263,482 | \$ 2,512,404 | \$ 2,533,935 |
| NINDS..... | \$ 1,187,046 | \$ 1,251,353 | \$ 1,191,401 | \$ 1,186,374 | \$ 1,199,197 |
| NIAID..... | \$ 3,339,295 | \$ 3,218,500 | \$ 3,272,795 | \$ 3,226,821 | \$ 3,320,733 |
| NIGMS..... | \$ 1,238,235 | \$ 1,059,416 | \$ 1,074,398 | \$ 1,340,000 | \$ 1,567,000 |
| NICHD..... | \$ 2,754,030 | \$ 2,294,903 | \$ 1,783,425 | \$ 1,609,294 | \$ 1,682,001 |
| NEI..... | \$ 2,429,549 | \$ 2,356,502 | \$ 2,354,021 | \$ 1,749,251 | \$ 1,665,234 |
| NIEHS..... | \$ 802,438 | \$ 940,172 | \$ 879,319 | \$ 1,005,525 | \$ 1,024,716 |
| NIA..... | \$ 1,530,068 | \$ 1,335,565 | \$ 1,232,260 | \$ 1,384,363 | \$ 1,323,099 |
| NIAMS..... | \$ 2,278,871 | \$ 2,442,115 | \$ 2,547,684 | \$ 2,314,357 | \$ 2,343,200 |
| NIDCD..... | \$ 1,474,638 | \$ 994,468 | \$ 1,125,546 | \$ 1,137,000 | \$ 1,137,000 |
| NIMH..... | \$ 1,172,325 | \$ 1,176,302 | \$ 1,141,553 | \$ 1,146,740 | \$ 1,152,069 |
| NIDA..... | \$ 1,658,432 | \$ 1,790,274 | \$ 1,649,930 | \$ 1,647,371 | \$ 1,778,411 |
| NIAAA..... | \$ 1,636,120 | \$ 1,673,276 | \$ 1,814,281 | \$ 2,452,650 | \$ 2,520,882 |
| NINR..... | \$ 1,003,205 | \$ 906,428 | \$ 980,570 | \$ 910,382 | \$ 913,432 |
| NHGRI..... | \$ 1,715,355 | \$ 1,571,612 | \$ 1,252,439 | \$ 1,264,828 | \$ 1,289,000 |
| NIBIB..... | \$ 1,499,663 | \$ 1,719,357 | \$ 1,268,882 | \$ 1,191,400 | \$ 1,310,304 |
| NIMHD..... | \$ 738,604 | \$ 991,360 | \$ 1,085,749 | \$ 885,749 | \$ 576,509 |
| NCCAM..... | \$ 1,085,753 | \$ 1,218,867 | \$ 1,393,027 | \$ 1,429,917 | \$ 1,507,039 |
| NCATS..... | N/A | \$ 546,927 | \$ 1,123,640 | \$ 1,929,939 | \$ 2,298,897 |
| FIC..... | \$ 1,478,739 | \$ 1,751,076 | \$ 1,549,305 | \$ 1,600,980 | \$ 1,611,912 |
| NLM..... | \$ 2,034,885 | \$ 2,267,557 | \$ 2,398,780 | \$ 2,432,641 | \$ 2,466,151 |
| CSR..... | \$ 1,961,774 | \$ 1,478,715 | \$ 1,038,012 | \$ 1,145,825 | \$ 1,156,304 |
| CIT..... | \$ 1,428,427 | \$ 1,138,841 | \$ 1,823,848 | \$ 1,882,931 | \$ 1,810,176 |
| CC..... | \$ 3,214,975 | \$ 3,948,197 | \$ 3,510,647 | \$ 3,660,135 | \$ 3,708,056 |
| OD..... | \$ 4,232,961 | \$ 3,302,110 | \$ 2,989,628 | \$ 1,996,298 | \$ 1,996,298 |
| Total..... | \$ 52,079,937 | \$ 50,510,259 | \$ 48,949,281 | \$ 49,491,392 | \$ 50,524,872 |

IC Director's Office Cost Summary
(Amounts in Dollars)

| FY 2011 | Personnel Compensation & Benefits - Subtotal | <i>Cash Awards & Performance Bonus (Non-add)</i> | Travel & Transportation of Persons | All Other | |
|-------------------|---|--|---|----------------------|----------------------|
| | OC 11, 12 and 13 | OC 115x | OC 21 | OC | Total |
| NCL..... | \$ 2,499,396 | \$ 96,437 | \$ 194,458 | \$ 2,344,187 | \$ 5,038,041 |
| NHLBI..... | \$ 1,474,886 | \$ 57,673 | \$ 33,453 | \$ 36,341 | \$ 1,544,680 |
| NIDCR..... | \$ 422,379 | \$ 15,907 | \$ 10,041 | \$ 389,131 | \$ 821,551 |
| NIDDK..... | \$ 1,522,200 | \$ 72,446 | \$ 129,842 | \$ 1,128,234 | \$ 2,780,276 |
| NINDS..... | \$ 884,926 | \$ 44,411 | \$ 99,637 | \$ 202,483 | \$ 1,187,046 |
| NIAID..... | \$ 2,765,672 | \$ 134,947 | \$ 118,361 | \$ 455,262 | \$ 3,339,295 |
| NIGMS..... | \$ 958,086 | \$ 30,953 | \$ 20,057 | \$ 260,092 | \$ 1,238,235 |
| NICHD..... | \$ 2,251,810 | \$ 61,435 | \$ 176,594 | \$ 325,626 | \$ 2,754,030 |
| NEI..... | \$ 1,424,180 | \$ 56,482 | \$ 95,400 | \$ 909,970 | \$ 2,429,549 |
| NIEHS..... | \$ 656,899 | \$ 8,896 | \$ 59,665 | \$ 85,874 | \$ 802,438 |
| NIA..... | \$ 1,454,439 | \$ 52,264 | \$ 57,134 | \$ 18,495 | \$ 1,530,068 |
| NIAMS..... | \$ 1,655,555 | \$ 130,539 | \$ 56,402 | \$ 566,914 | \$ 2,278,871 |
| NIDCD..... | \$ 1,029,666 | \$ 64,173 | \$ 10,468 | \$ 434,504 | \$ 1,474,638 |
| NIMH..... | \$ 997,642 | \$ 40,697 | \$ 82,308 | \$ 92,375 | \$ 1,172,325 |
| NIDA..... | \$ 1,532,591 | \$ 58,378 | \$ 69,377 | \$ 56,465 | \$ 1,658,432 |
| NIAAA..... | \$ 1,486,308 | \$ 49,952 | \$ 79,041 | \$ 70,771 | \$ 1,636,120 |
| NINR..... | \$ 760,101 | \$ 36,205 | \$ 35,816 | \$ 207,288 | \$ 1,003,205 |
| NHGRI..... | \$ 1,070,401 | \$ 28,650 | \$ 76,650 | \$ 568,304 | \$ 1,715,355 |
| NIBIB..... | \$ 999,087 | \$ 42,828 | \$ 124,094 | \$ 376,481 | \$ 1,499,663 |
| NIMHD..... | \$ 621,070 | \$ 22,535 | \$ 10,911 | \$ 106,624 | \$ 738,604 |
| NCCAM..... | \$ 859,442 | \$ 42,328 | \$ 33,774 | \$ 192,537 | \$ 1,085,753 |
| NCATS..... | N/A | N/A | N/A | N/A | N/A |
| FIC..... | \$ 1,180,049 | \$ 34,783 | \$ 120,611 | \$ 178,079 | \$ 1,478,739 |
| NLM..... | \$ 1,465,244 | \$ 68,114 | \$ 176,268 | \$ 393,373 | \$ 2,034,885 |
| CSR..... | \$ 1,283,133 | \$ 35,518 | \$ 64,581 | \$ 614,060 | \$ 1,961,774 |
| CIT..... | \$ 526,902 | \$ 26,212 | \$ 7,517 | \$ 894,008 | \$ 1,428,427 |
| CC..... | \$ 1,835,918 | \$ 60,040 | \$ 64,215 | \$ 1,314,842 | \$ 3,214,975 |
| OD..... | \$ 3,101,439 | \$ 96,115 | \$ 99,844 | \$ 1,031,678 | \$ 4,232,961 |
| Total..... | \$ 36,719,420 | \$ 1,468,918 | \$ 2,106,520 | \$ 13,253,997 | \$ 52,079,937 |

IC Director's Office Cost Summary
(Amounts in Dollars)

| FY 2012 | Personnel Compensation & Benefits - Subtotal | Cash Awards & Performance Bonus (Non-add) | Travel & Transportation of Persons | All Other | Total |
|-------------------|---|--|---|----------------------|----------------------|
| | OC 11, 12 and 13 | OC 115x | OC 21 | OC | |
| NCI..... | \$ 2,391,323 | \$ 38,088 | \$ 92,964 | \$ 1,816,864 | \$ 4,301,151 |
| NHLBI..... | \$ 1,187,992 | \$ 20,581 | \$ 49,244 | \$ 45,631 | \$ 1,282,867 |
| NIDCR..... | \$ 848,605 | \$ 9,146 | \$ 14,159 | \$ 194,226 | \$ 1,056,990 |
| NIDDK..... | \$ 1,540,240 | \$ 43,118 | \$ 99,810 | \$ 855,308 | \$ 2,495,358 |
| NINDS..... | \$ 915,107 | \$ 31,603 | \$ 71,565 | \$ 264,681 | \$ 1,251,353 |
| NIAID..... | \$ 2,760,624 | \$ 70,514 | \$ 56,357 | \$ 401,519 | \$ 3,218,500 |
| NIGMS..... | \$ 859,668 | \$ 7,418 | \$ 9,735 | \$ 190,013 | \$ 1,059,416 |
| NICHD..... | \$ 1,941,195 | \$ 37,950 | \$ 64,418 | \$ 289,290 | \$ 2,294,903 |
| NEI..... | \$ 1,486,277 | \$ 31,079 | \$ 101,157 | \$ 769,068 | \$ 2,356,502 |
| NIEHS..... | \$ 821,840 | \$ 8,594 | \$ 53,672 | \$ 64,660 | \$ 940,172 |
| NIA..... | \$ 1,283,483 | \$ 34,737 | \$ 15,122 | \$ 36,960 | \$ 1,335,565 |
| NIAMS..... | \$ 1,668,726 | \$ 74,892 | \$ 38,370 | \$ 735,019 | \$ 2,442,115 |
| NIDCD..... | \$ 953,162 | \$ 32,848 | \$ 288 | \$ 41,018 | \$ 994,468 |
| NIMH..... | \$ 1,008,317 | \$ 36,941 | \$ 51,960 | \$ 116,025 | \$ 1,176,302 |
| NIDA..... | \$ 1,655,765 | \$ 46,221 | \$ 69,623 | \$ 64,886 | \$ 1,790,274 |
| NIAAA..... | \$ 1,464,446 | \$ 27,629 | \$ 116,594 | \$ 92,235 | \$ 1,673,276 |
| NINR..... | \$ 697,899 | \$ 10,045 | \$ 29,205 | \$ 179,324 | \$ 906,428 |
| NHGRI..... | \$ 1,005,240 | \$ 33,753 | \$ 76,907 | \$ 489,465 | \$ 1,571,612 |
| NIBIB..... | \$ 1,025,760 | \$ 25,718 | \$ 104,723 | \$ 588,874 | \$ 1,719,357 |
| NIMHD..... | \$ 863,750 | \$ 17,646 | \$ 14,374 | \$ 113,237 | \$ 991,360 |
| NCCAM..... | \$ 949,225 | \$ 40,987 | \$ 43,810 | \$ 225,832 | \$ 1,218,867 |
| NCATS..... | \$ 436,767 | \$ 45,419 | \$ 6,281 | \$ 103,879 | \$ 546,927 |
| FIC..... | \$ 1,373,310 | \$ 30,363 | \$ 124,995 | \$ 252,771 | \$ 1,751,076 |
| NLM..... | \$ 1,431,707 | \$ 36,809 | \$ 171,290 | \$ 664,560 | \$ 2,267,557 |
| CSR..... | \$ 967,186 | \$ 14,673 | \$ 45,848 | \$ 465,681 | \$ 1,478,715 |
| CIT..... | \$ 747,655 | \$ 10,672 | \$ 1,624 | \$ 389,562 | \$ 1,138,841 |
| CC..... | \$ 1,765,918 | \$ 38,791 | \$ 11,681 | \$ 2,170,598 | \$ 3,948,197 |
| OD..... | \$ 2,679,305 | \$ 76,925 | \$ 64,293 | \$ 558,512 | \$ 3,302,110 |
| Total..... | \$ 36,730,491 | \$ 933,160 | \$ 1,600,070 | \$ 12,179,697 | \$ 50,510,259 |

IC Director's Office Cost Summary
(Amounts in Dollars)

| FY 2013 | Personnel Compensation & Benefits - Subtotal | <i>Cash Awards & Performance Bonus (Non-add)</i> | Travel & Transportation of Persons | All Other | Total |
|-------------------|---|--|---|----------------------|----------------------|
| | OC 11, 12 and 13 | OC 115x | OC 21 | OC | |
| NCL..... | \$ 1,801,617 | \$ 13,000 | \$ 117,431 | \$ 1,546,613 | \$ 3,465,661 |
| NHLBI..... | \$ 1,630,589 | \$ 114,622 | \$ 34,101 | \$ 22,957 | \$ 1,687,648 |
| NIDCR..... | \$ 804,250 | \$ 13,653 | \$ 11,938 | \$ 235,162 | \$ 1,051,350 |
| NIDDK..... | \$ 1,396,545 | \$ 31,094 | \$ 44,615 | \$ 822,323 | \$ 2,263,482 |
| NINDS..... | \$ 956,177 | \$ 46,851 | \$ 46,213 | \$ 189,011 | \$ 1,191,401 |
| NIAID..... | \$ 2,773,136 | \$ 47,750 | \$ 86,539 | \$ 413,120 | \$ 3,272,795 |
| NIGMS..... | \$ 791,525 | \$ 600 | \$ 9,075 | \$ 273,798 | \$ 1,074,398 |
| NICHD..... | \$ 1,488,448 | \$ 34,778 | \$ 63,307 | \$ 231,670 | \$ 1,783,425 |
| NEL..... | \$ 1,335,075 | \$ 22,201 | \$ 121,111 | \$ 897,834 | \$ 2,354,021 |
| NIEHS..... | \$ 767,941 | \$ 9,637 | \$ 71,437 | \$ 39,941 | \$ 879,319 |
| NIA..... | \$ 1,199,224 | \$ 25,709 | \$ 19,550 | \$ 13,486 | \$ 1,232,260 |
| NIAMS..... | \$ 1,743,941 | \$ 36,500 | \$ 20,956 | \$ 782,787 | \$ 2,547,684 |
| NIDCD..... | \$ 916,479 | \$ 33,054 | \$ 5,595 | \$ 203,472 | \$ 1,125,546 |
| NIMH..... | \$ 1,016,300 | \$ 43,947 | \$ 38,232 | \$ 87,021 | \$ 1,141,553 |
| NIDA..... | \$ 1,502,258 | \$ 26,002 | \$ 97,076 | \$ 50,596 | \$ 1,649,930 |
| NIAAA..... | \$ 1,622,954 | \$ 13,304 | \$ 100,289 | \$ 91,038 | \$ 1,814,281 |
| NINR..... | \$ 853,242 | \$ 4,300 | \$ 26,114 | \$ 101,214 | \$ 980,570 |
| NHGRI..... | \$ 797,661 | \$ 25,773 | \$ 90,450 | \$ 364,328 | \$ 1,252,439 |
| NIBIB..... | \$ 744,199 | \$ 27,495 | \$ 51,530 | \$ 473,153 | \$ 1,268,882 |
| NIMHD..... | \$ 954,769 | \$ 25,523 | \$ 12,669 | \$ 118,311 | \$ 1,085,749 |
| NCCAM..... | \$ 1,164,153 | \$ 35,524 | \$ 13,256 | \$ 215,618 | \$ 1,393,027 |
| NCATS..... | \$ 911,452 | \$ 53,264 | \$ 51,876 | \$ 160,312 | \$ 1,123,640 |
| FIC..... | \$ 1,186,879 | \$ 19,828 | \$ 63,229 | \$ 299,197 | \$ 1,549,305 |
| NLM..... | \$ 1,430,244 | \$ 24,776 | \$ 73,973 | \$ 894,563 | \$ 2,398,780 |
| CSR..... | \$ 648,037 | \$ 10,056 | \$ 36,434 | \$ 353,541 | \$ 1,038,012 |
| CIT..... | \$ 828,898 | \$ 8,985 | \$ 3,273 | \$ 991,676 | \$ 1,823,848 |
| CC..... | \$ 1,725,903 | \$ 29,076 | \$ 9,595 | \$ 1,775,149 | \$ 3,510,647 |
| OD..... | \$ 2,441,303 | \$ 64,430 | \$ 48,399 | \$ 499,927 | \$ 2,989,628 |
| Total..... | \$ 35,433,199 | \$ 841,731 | \$ 1,368,264 | \$ 12,147,818 | \$ 48,949,281 |

IC Director's Office Cost Summary
(Amounts in Dollars)

| FY 2014 (est.) | Personnel Compensation & Benefits - Subtotal | Cash Awards & Performance Bonus (Non-add) | Travel & Transportation of Persons | All Other | Total |
|-----------------------|---|--|---|----------------------|----------------------|
| | OC 11, 12 and 13 | OC 115x | OC 21 | OC | |
| NCI..... | \$ 1,877,816 | \$ 21,069 | \$ 117,000 | \$ 1,500,000 | \$ 3,494,816 |
| NHLBI..... | \$ 1,646,895 | \$ 114,622 | \$ 34,101 | \$ 22,957 | \$ 1,703,954 |
| NIDCR..... | \$ 996,847 | \$ 7,303 | \$ 15,100 | \$ 237,500 | \$ 1,249,447 |
| NIDDK..... | \$ 1,546,329 | \$ 48,520 | \$ 80,000 | \$ 886,075 | \$ 2,512,404 |
| NINDS..... | \$ 947,622 | \$ 34,243 | \$ 46,906 | \$ 191,846 | \$ 1,186,374 |
| NIAID..... | \$ 2,727,137 | \$ 48,022 | \$ 86,556 | \$ 413,128 | \$ 3,226,821 |
| NIGMS..... | \$ 1,048,000 | \$ 10,000 | \$ 10,000 | \$ 282,000 | \$ 1,340,000 |
| NICHD..... | \$ 1,312,894 | \$ 57,600 | \$ 64,400 | \$ 232,000 | \$ 1,609,294 |
| NEI..... | \$ 1,319,259 | \$ 25,611 | \$ 85,508 | \$ 344,484 | \$ 1,749,251 |
| NIHES..... | \$ 842,780 | \$ 10,028 | \$ 72,508 | \$ 90,237 | \$ 1,005,525 |
| NIA..... | \$ 1,325,559 | \$ 28,874 | \$ 13,665 | \$ 45,139 | \$ 1,384,363 |
| NIAMS..... | \$ 1,684,357 | \$ 55,000 | \$ 30,000 | \$ 600,000 | \$ 2,314,357 |
| NIDCD..... | \$ 920,000 | \$ 33,000 | \$ 7,000 | \$ 210,000 | \$ 1,137,000 |
| NIMH..... | \$ 1,019,609 | \$ 43,947 | \$ 38,805 | \$ 88,326 | \$ 1,146,740 |
| NIDA..... | \$ 1,462,997 | \$ 35,692 | \$ 107,000 | \$ 77,374 | \$ 1,647,371 |
| NIAAA..... | \$ 2,123,071 | \$ 27,282 | \$ 181,073 | \$ 148,506 | \$ 2,452,650 |
| NINR..... | \$ 781,536 | \$ 6,380 | \$ 26,114 | \$ 102,732 | \$ 910,382 |
| NHGRI..... | \$ 813,614 | \$ 26,288 | \$ 79,214 | \$ 372,000 | \$ 1,264,828 |
| NIBIB..... | \$ 547,249 | \$ 27,340 | \$ 80,000 | \$ 564,151 | \$ 1,191,400 |
| NIMHD..... | \$ 571,554 | \$ 11,677 | \$ 22,859 | \$ 291,336 | \$ 885,749 |
| NCCAM..... | \$ 1,197,610 | \$ 35,524 | \$ 13,455 | \$ 218,852 | \$ 1,429,917 |
| NCATS..... | \$ 1,541,391 | \$ 57,344 | \$ 78,828 | \$ 309,720 | \$ 1,929,939 |
| FIC..... | \$ 1,233,117 | \$ 24,856 | \$ 64,178 | \$ 303,685 | \$ 1,600,980 |
| NLM..... | \$ 1,450,687 | \$ 26,110 | \$ 73,973 | \$ 907,981 | \$ 2,432,641 |
| CSR..... | \$ 750,000 | \$ 12,277 | \$ 36,980 | \$ 358,844 | \$ 1,145,825 |
| CIT..... | \$ 979,008 | \$ 17,093 | \$ 3,923 | \$ 900,000 | \$ 1,882,931 |
| CC..... | \$ 1,848,620 | \$ 29,076 | \$ 9,739 | \$ 1,801,776 | \$ 3,660,135 |
| OD..... | \$ 1,499,106 | \$ 39,182 | \$ 48,399 | \$ 448,793 | \$ 1,996,298 |
| Total..... | \$ 36,014,665 | \$ 913,960 | \$ 1,527,285 | \$ 11,949,442 | \$ 49,491,392 |

IC Director's Office Cost Summary
(Amounts in Dollars)

| FY 2015 (est.) | Personnel Compensation & Benefits - Subtotal | <i>Cash Awards & Performance Bonus (Non-add)</i> | Travel & Transportation of Persons | All Other | |
|-----------------------|---|--|---|----------------------|----------------------|
| | OC 11, 12 and 13 | OC 115x | OC 21 | OC | Total |
| NCI..... | \$ 1,896,594 | \$ 21,000 | \$ 112,000 | \$ 1,500,000 | \$ 3,508,594 |
| NHLBI..... | \$ 1,663,364 | \$ 114,622 | \$ 34,101 | \$ 22,957 | \$ 1,720,423 |
| NIDCR..... | \$ 1,143,000 | \$ 10,000 | \$ 15,300 | \$ 246,000 | \$ 1,404,300 |
| NIDDK..... | \$ 1,555,683 | \$ 49,286 | \$ 80,850 | \$ 897,402 | \$ 2,533,935 |
| NINDS..... | \$ 956,387 | \$ 34,000 | \$ 47,703 | \$ 195,107 | \$ 1,199,197 |
| NIAID..... | \$ 2,821,019 | \$ 48,000 | \$ 86,575 | \$ 413,139 | \$ 3,320,733 |
| NIGMS..... | \$ 1,270,000 | \$ 20,000 | \$ 10,000 | \$ 287,000 | \$ 1,567,000 |
| NICHD..... | \$ 1,385,601 | \$ 57,600 | \$ 64,400 | \$ 232,000 | \$ 1,682,001 |
| NEI..... | \$ 1,229,386 | \$ 25,611 | \$ 85,508 | \$ 350,340 | \$ 1,665,234 |
| NIEHS..... | \$ 921,497 | \$ 10,128 | \$ 73,741 | \$ 29,478 | \$ 1,024,716 |
| NIA..... | \$ 1,293,795 | \$ 28,874 | \$ 13,665 | \$ 15,639 | \$ 1,323,099 |
| NIAMS..... | \$ 1,701,200 | \$ 55,550 | \$ 30,000 | \$ 612,000 | \$ 2,343,200 |
| NIDCD..... | \$ 925,000 | \$ 33,000 | \$ 7,000 | \$ 205,000 | \$ 1,137,000 |
| NIMH..... | \$ 1,022,776 | \$ 43,947 | \$ 39,465 | \$ 89,828 | \$ 1,152,069 |
| NIDA..... | \$ 1,593,941 | \$ 36,227 | \$ 107,000 | \$ 77,470 | \$ 1,778,411 |
| NIAAA..... | \$ 2,188,851 | \$ 41,532 | \$ 181,000 | \$ 151,031 | \$ 2,520,882 |
| NINR..... | \$ 782,296 | \$ 6,380 | \$ 26,658 | \$ 104,478 | \$ 913,432 |
| NHGRI..... | \$ 830,000 | \$ 26,288 | \$ 80,000 | \$ 379,000 | \$ 1,289,000 |
| NIBIB..... | \$ 666,153 | \$ 27,340 | \$ 80,000 | \$ 564,151 | \$ 1,310,304 |
| NIMHD..... | \$ 358,179 | \$ 7,048 | \$ 18,078 | \$ 200,252 | \$ 576,509 |
| NCCAM..... | \$ 1,274,274 | \$ 35,524 | \$ 13,481 | \$ 219,284 | \$ 1,507,039 |
| NCATS..... | \$ 1,858,897 | \$ 58,000 | \$ 110,000 | \$ 330,000 | \$ 2,298,897 |
| FIC..... | \$ 1,237,796 | \$ 30,000 | \$ 65,269 | \$ 308,847 | \$ 1,611,912 |
| NLM..... | \$ 1,468,761 | \$ 26,110 | \$ 73,973 | \$ 923,417 | \$ 2,466,151 |
| CSR..... | \$ 753,750 | \$ 15,000 | \$ 37,609 | \$ 364,945 | \$ 1,156,304 |
| CIT..... | \$ 900,276 | \$ 19,000 | \$ 4,900 | \$ 905,000 | \$ 1,810,176 |
| CC..... | \$ 1,865,745 | \$ 29,076 | \$ 9,905 | \$ 1,832,406 | \$ 3,708,056 |
| OD..... | \$ 1,514,356 | \$ 39,182 | \$ 48,399 | \$ 433,543 | \$ 1,996,298 |
| Total..... | \$ 37,078,578 | \$ 948,326 | \$ 1,556,580 | \$ 11,889,714 | \$ 50,524,872 |

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